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IN THE UNITED STATES PATENT AND TRADEMARK OFFICE  
BEFORE THE TRADEMARK TRIAL AND APPEAL BOARD

Proceeding	91247208
Party	Plaintiff MedImmune, LLC
Correspondence Address	DAVID M KELLY KELLY IP LLP 1300 19TH STREET NW, SUITE 300 WASHINGTON, DC 20036 UNITED STATES Primary Email: david.kelly@kelly-ip.com Secondary Email(s): sabrina.shyn@kelly-ip.com, lit-docketing@kelly-ip.com 202-808-3570
Submission	Testimony For Plaintiff
Filer's Name	DAVID M. KELLY
Filer's email	david.kelly@kelly-ip.com, jason.joyal@kelly-ip.com, lit-docketing@kelly-ip.com
Signature	/David M. Kelly/
Date	10/13/2020
Attachments	Group 5 Cover.pdf(16738 bytes ) Exhibit 10-1.pdf(3688207 bytes ) Exhibit 10-2.pdf(4274410 bytes ) Exhibit 10-3.pdf(3819747 bytes ) Exhibit 10-4.pdf(1951042 bytes ) Exhibit 10-5.pdf(2039739 bytes ) Exhibit 10-6.pdf(5220659 bytes )

**IN THE UNITED STATES PATENT AND TRADEMARK OFFICE  
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<p>MEDIMMUNE, LLC,</p> <p style="text-align: right;">Opposer,</p> <p style="text-align: center;">v.</p> <p>PRASUN J. MISHRA, PHD,</p> <p style="text-align: right;">Applicant.</p>	<p>Opposition No.: 91247208</p> <p style="text-align: center;"></p> <p>Mark: Serial No.: 87953597 Filed: June 7, 2018</p>
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**DECLARATION OF RICHARD BUCKLEY**

**EXHIBIT 10**

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- microRNA pathways in disease
- microRNA therapeutics: design and delivery
- Role of microRNA in viral infection
- Therapeutic potential of microRNA
- Role of microRNA in cancer mechanism
- microRNA as targets in therapeutic development

### Distinguished Speaker List

C.D. Atreya, Ph.D., Associate Director for Research, Office of Blood Research and Review, CBER, FDA

Brian D. Brown, Ph.D., Assistant Professor, Genetics and Genomic Sciences, Mount Sinai School of Medicine

Youhai H. Chen, M.D., Ph.D., Professor, Pathology and Lab Medicine, University of Pennsylvania School of Medicine

Donald Coen, Ph.D., Professor, Biological Chemistry and Molecular Pharmacology, Harvard Medical School

Matthew J. Evans, Ph.D., Assistant Professor, Microbiology, Mount Sinai School of Medicine

Joshua Friedman, M.D., Ph.D., Assistant Professor, Pediatrics, Perelman School of Medicine, University of Pennsylvania

Richard I. Gregory, Ph.D., Assistant Professor, Children's Hospital Boston, Harvard Stem Cell Institute (HSCI), Harvard Medical School

Lin He, Ph.D., Assistant Professor, Cell and Developmental Biology, University of California Berkeley

Eva Hernando, Ph.D., Assistant Professor, Pathology, New York University School of Medicine

Clark D. Jeffries, Ph.D., Research Professor, UNC Eshelman School of Pharmacy and Renaissance Computing Institute, University of North Carolina at Chapel Hill

Feng Jiang, M.D., Ph.D., Associate Professor, Pathology, University of Maryland School of Medicine

Jingfang Ju, Ph.D., Professor and Co-Director, Translational Research Laboratory, Pathology, State University of New York at Stony Brook

Andrea L. Kasinski, Ph.D., Postdoctoral Fellow, Frank Slack's Lab, Molecular, Cellular and Developmental Biology, Yale University

Mark A. Kay, M.D., Ph.D., Dennis Farrey Family Professor, Pediatrics and Genetics; Vice Chair for Basic Research, Pediatrics, Stanford University

Debomoy K. Lahiri, Ph.D., Professor of Neuroscience, Departments of Psychiatry and Medical and Molecular Genetics; Member, Stark Neurosciences Research Institute, Indiana University School of Medicine Institute of Psychiatric Research

Walter J. Lukiw, B.S., M.S., Ph.D., Principal Investigator, LSU Neuroscience Center and Department of Ophthalmology, Louisiana State University Health Sciences Center

Muthiah Manoharan, Ph.D., Vice President, Drug Discovery, Alnylam

Julie Mathieu, Ph.D., Postdoctoral Fellow, Biochemistry, University of Washington

Prasun J. Mishra, Ph.D., Laboratory of Cancer Biology and Genetics, National Cancer Institute, NIH

Benjamin Ory, Ph.D., Associate Professor, Physiopathology of Bone Resorption, Nantes Medical School, INSERM UMR957, France

Bulent Ozpolat, M.D., Ph.D., Assistant Professor, Experimental Therapeutics, University of Texas MD Anderson Cancer Center

Igor Pogribny, M.D., Ph.D., Laboratory Director and Principal Research Investigator, Division of Biochemical Toxicology, National Center for Toxicological Research, U.S. Food and Drug Administration; Basic Science Professor, Pharmacology and Toxicology, University of Arkansas Medical Sciences

Tariq M. Rana, Ph.D., Professor and Director, Program for RNA Biology, Sanford-Burnham Medical Research Institute

Fazlul H. Sarkar, Ph.D., Professor of Pathology, Barbara Ann Karmanos Cancer Institute, Wayne State University School of Medicine

Thomas D. Schmittgen, Ph.D., Associate Professor and Chair, Division of Pharmaceuticals, Ohio State University

Lorenzo F. Sempere, Ph.D., Research Assistant Professor, Medicine, Dartmouth-Hitchcock/Norris Cotton Cancer Center

Søren P. Sheikh, M.D., Ph.D., Professor and Head, Biochemistry, Pharmacology, and Genetics, Odense University Hospital

Katie Streicher, Ph.D., Scientist II, Translational Science, MedImmune

Tao Sun, Ph.D., Associate Professor, Cell and Developmental Biology, Weill Medical College of Cornell University

Andrei Thomas-Tikhonenko, Ph.D., Chief, Division of Cancer Pathobiology, The Children's Hospital of Philadelphia

Philip N. Tsichlis, M.D., Jane F. Desforges Professor of Hematology and Oncology; Executive Director, Medical Oncology Research Institute, Tufts Medical Center

Scott J. Valastyan, Ph.D., Research Fellow, Cell Biology, Harvard Medical School

Eva van Rooij, Ph.D., Senior Director of Biology, Miragen Therapeutics

David von Schack, Ph.D., Senior Principal Research Scientist, Translational Immunology, Pfizer

Joanne Weidhaas, M.D., Ph.D., Assistant Professor, Therapeutic Radiology, Yale University School of Medicine

Glen J. Weiss, M.D., Co-Head, Lung Cancer Unit, The Translational Genomics Research Institute (TGen); Director, Thoracic Oncology, Virginia G. Piper Cancer Center Clinical Trials at Scottsdale Healthcare; CMO, CRAB-Clinical Trials Consortium









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# Insights from Merck, Amgen, Novartis, MedImmune and Lonza on Immuno-Oncology

Published on December 29, 2016



**Amit Shelke**  
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Research community is already calling immunotherapies the ‘**fifth pillar**’ of cancer treatment as they harness the power of a patient’s immune system paving the way for emerging next generation therapeutics. The **Next Gen Immuno-Oncology Congress** taking place in **London** on **14th – 15th March 2017**, will bring together experts from the immune-oncology space, to provide a platform for industry experts to gather and collaborate to further more developments.

With participation from **Merck, Amgen, Novartis, MedImmune, Lonza** and many more leading companies, this meeting will look at the challenges faced by experts in immune-oncology and how best to overcome them, aiding efforts in cancer treatment and therapies.

The conference is divided into three streams, these are dedicated to antibody drug conjugates, bispecific antibodies and immune checkpoint inhibitors to enhance target identification, improve preclinical development, overcome manufacturing challenges and maximize clinical performance.

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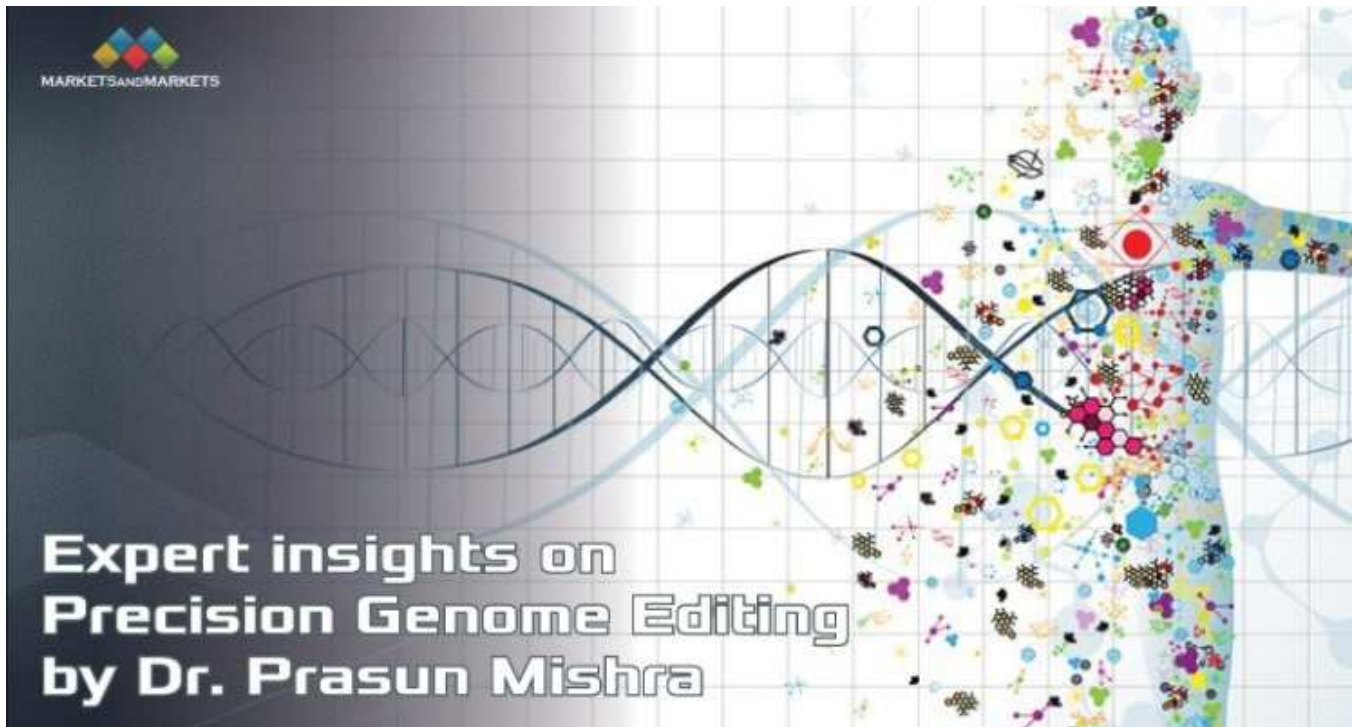
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## Prasun Mishra, Founder & CEO of Agility Pharmaceuticals talks about recent advances & analytics contribution in genome editing technologies

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**Dr. Prasun Mishra - Founder and CEO of the Agility Pharmaceuticals** recently spoke to MarketsandMarket's team sharing the valuable insights into the industry with respect to emerging areas, challenges and innovations. Dr. Mishra (Ex-Genentech, Ex-NCI/NIH) has been leading Agility Pharmaceuticals, a pharmaceutical company driven by artificial intelligence (AI) to revolutionize drug discovery through BigData, Robotics and AI. He is the founding president and CEO of American Association for Precision Medicine (AAPM), one of the world's leading professional organizations dedicated to advancing the field of precision medicine. We interviewed Dr. Mishra asking about his expert insights on the genome editing space, his presence at the 3rd Annual Genome Editing & Engineering Conference and his take on the conference program.

**Q: What are the current emerging areas of Genome Editing?**

Dr. Mishra:

Recent advances in genome editing technologies have taken the scientific community by storm, working their way into virtually every corner of biological inquiry, including human health. Advances to human health by gene editing applications can be summarized in following four categories:

From basic research to translational applications: Gene editing of cells, tissues, germline cells has provided a deeper understanding of human biology and disease mechanisms that has pioneered new approaches of treatment. Discovery of CRISPR-Cas9 system for genetic manipulation has been one of the most exciting developments in recent times which is already redefining our approach in identifying new drugs/targets

Somatic cell genome editing to prevent/treat human diseases and disorders: Human genome editing in somatic cells, to treat/prevent a variety of genetically inherited diseases, has an immediate clinical application. This approach is already in clinical trials for several indications including to treat children with the severe combined immune deficiency or "bubble baby" disease, now in a clinic (De Ravin et al., 2016) as well as to restore hearing in mice (Pan et al., 2017, Nature Biotechnology). Recent FDA approval of tisagenlecleucel for acute lymphoblastic leukemia and axicabtagene ciloleucel for lymphoma brought gene therapy to clinic in the US

Heritable genome editing to prevent/alleviate the suffering caused by genetically inherited diseases: Gene editing presents an opportunity to prevent thousands of genetically inherited diseases and can be used as a potential "surgical knife" to correct genetic mutations. For example gene editing technology was used to correct a gene mutation in human embryos

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**Enhancements:** Gene editing can also be supplemental to human health, including enhancing human capabilities further. However, there are ethical concerns that "unregulated genetic engineering may lead to a new form of eugenics, in which people with means pay to have children with enhanced traits even as those with disabilities are devalued (Belluck, 2017, NYTimes)."

(<https://www.nytimes.com/2017/08/02/science/gene-editing-human-embryos.html>)

There are also safety concerns associated with gene editing technology raised by a recent study that attempted to restore sight in blind mice, using CRISPR-Cas9, observed "unexpected mutations" in secondary genes (Schaefer et al., 2017, Nature Methods). Hence, safety and efficacy concerns should be carefully addressed in human clinical trials employing gene editing applications.

**Q: For gene editing, analytics plays a vital part. How do you think analytics contributes in gene editing applications?**

Dr. Mishra:

Data analytics tools are a key in optimizing each step of genome editing experiments regardless of the genome-editing technique utilized. Briefly, a careful monitoring and analytics of the gene editing process starting with cell culture step (accurate cell counts, viability counts & measure apoptosis), the actual genome editing step (measuring delivery of lentivirus, Cas9, guide RNA as well as confirmation of DNA cleavage and gene editing) to the cell phenotyping step (primary target and off-target phenotyping) contributes to efficient gene editing results. Moreover, several next-generation sequencing data analytics tools have made the sequence level verification of guide RNA, primary target and off-target phenotyping analytics steps more effective. Although all these steps can be analyzed separately, there are still opportunities to innovate around integrating data analytic applications for the entire gene editing process.

**Q: You will be speaking at the 3rd Annual Genome Editing and Engineering Conference, could you please elaborate on few take away points from your presentation topic which will help in building the knowledge base for attendees?**

Dr. Mishra:

I am excited to discuss innovative solutions for pharmaceutical and healthcare industry using artificial intelligence. The title of my talk is "***Augmenting Human Intelligence through Artificial Intelligence.***" I will focus on how we can use accelerate drug discovery process using big data analytics, robotic screens and artificial intelligence (AI). Briefly, I will discuss how big data analytics can be utilized to make complex biology simpler. Moreover, I will also emphasize the importance of automation in drug discovery process and how we can use genotypic and phenotypic screening to understand high throughput biology. I also hope to give the audience an overview of recent advances in AI field and how we can exploit it for drug discovery and precision medicine.

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**“This conference brings together some of the great minds in field from both industry and academia in Genome editing”**

**Q: After seeing the Agenda for 3rd Annual Genome Editing and Engineering Conference, what are your views and how helpful do you think it is for the targeted audience?**

Dr. Mishra:

The agenda for the Genome Editing & Engineering Conference looks very interesting and I am looking forward to meeting old friends and making new ones. This conference brings together some of the great minds in the field from both industry and academia discussing genome editing. Among many interesting talks, the speakers will also discuss CRISPR screens (to better understand biology and drug discovery applications), data analytics, gene activation, efficient animal genome engineering and improving the accuracy of gene editing methods. The audience would also gain knowledge in clinical applications of genome editing in muscular dystrophy, friedreich, microsatellite diseases as well as therapeutic genome editing of hematopoietic stem cells. Those who are interested in immunotherapy would learn about the production of allogeneic CAR-T cells and its applications in adaptive allogeneic T-cell immunotherapy.

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presenting on the Day 1 of the conference on the topics - Augmenting Human Intelligence through Artificial Intelligence. Know more about his presentation visit our website -

<http://bit.ly/2hYF8sQ>

If you wish to learn a great deal from him over the insights of Drug Discovery, then register now online at <http://bit.ly/2zqdXRv> or email [amit.shelke@marketsandmarkets.com](mailto:amit.shelke@marketsandmarkets.com) to book your registration.

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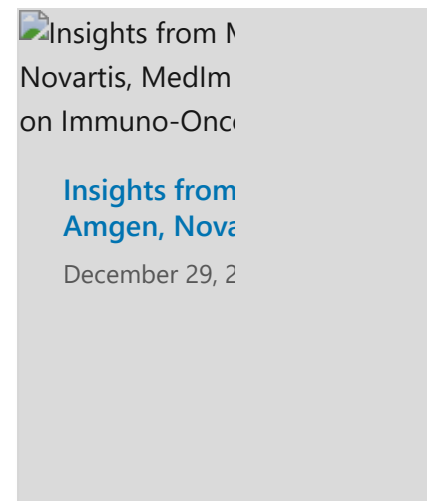
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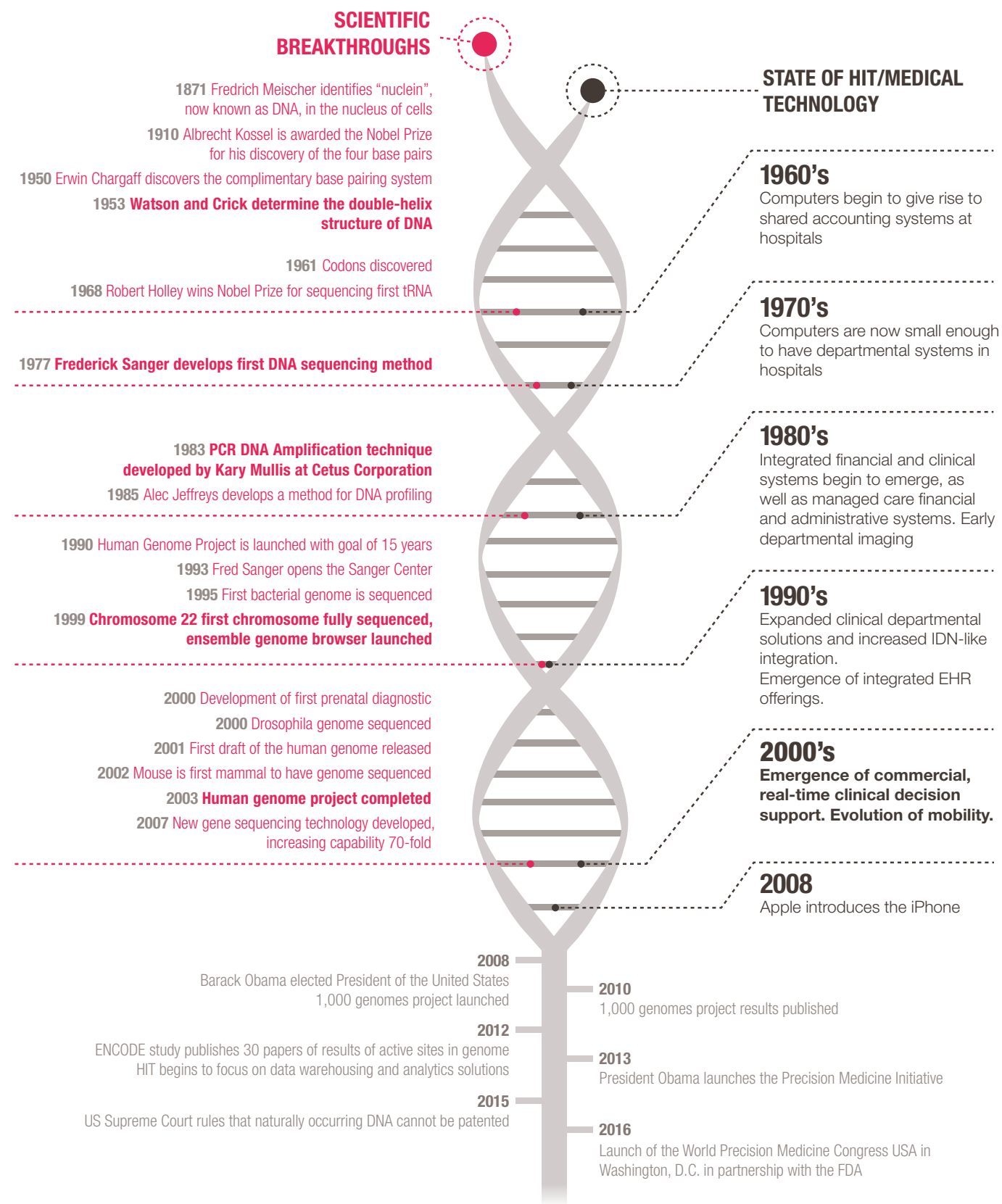
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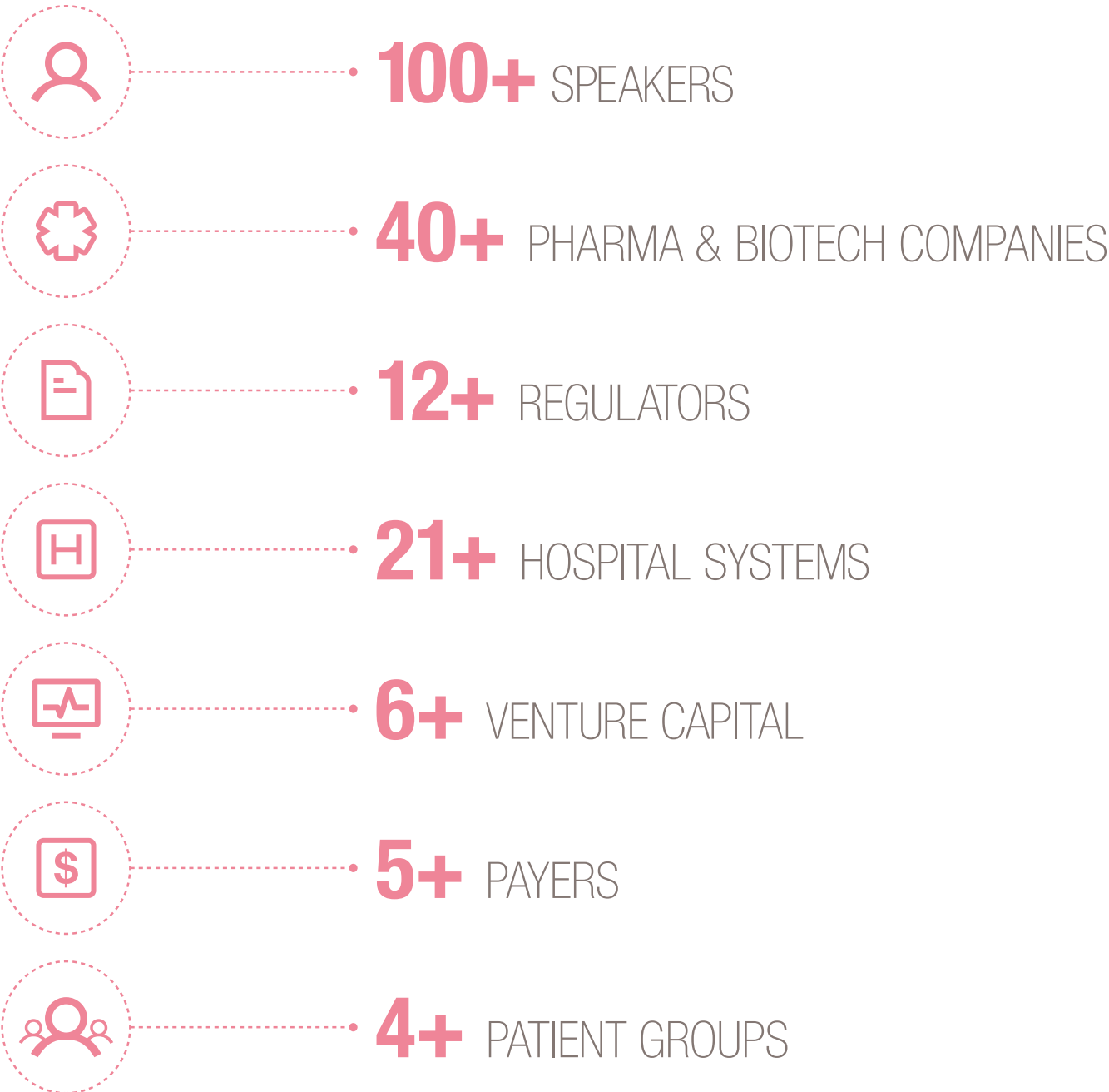
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**Mark Rubin**  
Founding Director  
Englander Institute for Precision Medicine  
**WEILL CORNELL MEDICAL COLLEGE**



**Fred Lorey**  
Voting Member, HHS Secretary's Advisory Committee on Heritable Disorders in Newborns and Children  
**CALIFORNIA HEALTH AND HUMAN SERVICES AGENCY**



**Fred Meindl**  
Sales Director  
**PERKINELMER**

# INDUSTRY LEADERS LEADING THE PRECISION MEDICINE REVOLUTION ARE SPEAKING AT WORLD PRECISION MEDICINE CONGRESS USA 2016



**Omar Ali**  
NHS Pharmacy Payer NICE  
**N.I.C.E. NATIONAL INSTITUTE FOR HEALTH AND CLINICAL EXCELLENCE**



**Elizabeth Ottinger**  
Senior Project Manager  
**NIH**



**David Roth**  
Director, Precision Medicine Program  
**PERELMAN SCHOOL OF MEDICINE UNIVERSITY OF PENNSYLVANIA**



**Kenna Mills Shaw**  
Executive Director and Institute For Personalized Cancer Therapy  
**M.D. ANDERSON CANCER CENTER**



**Anup Kaneri**  
Portfolio Strategist  
**UNIVERSITY OF PITTSBURGH MEDICAL CENTER**



**Sam Johnson**  
Director, Health Policy and Interprofessional Affairs  
**AMERICAN COLLEGE OF CLINICAL PHARMACY INC**



**Joshua Resnick**  
Biotechnology Partner  
**S.V. LIFE SCIENCES**



**Chris Bardon**  
Managing Director  
**MPM Oncology Impact Fund**



**Jit Patel**  
Vice President  
**J.D.R.F.**



**Kurtis Bachman**  
US Head, Computational and Systems Biology  
**JANSSEN**



**Mark Clein**  
Co-Founder, Chief Executive Officer  
**PRECISION FOR MEDICINE**



**Michelle Penny**  
Director Computational Biology and Genomics  
**BIOGEN**



**Richard Hockett**  
Chief Medical Officer  
**AFFYMETRIX**



**Eric Lai**  
Senior Vice President, Head of Pharmacogenomics & Companion Diagnostics  
**TAKEDA**



**Mitchell Martin**  
Director, Predictive Medicine, Oncology  
**REGENERON**



**Michael Murray**  
Director of Government Business Development  
**SOUTHERN RESEARCH INSTITUTE**



**Peter Shaw**  
Senior Director, Medical Affairs  
**FERRING PHARMACEUTICALS**



**Carolyn Jones**  
Director of Regulatory Policy  
**BIOGEN**



**Sarah Nia**  
Senior Director Clinical Sciences  
**ALEXION**



**Julie Venners Christensen**  
Head of Global Patient Advocacy for Gene Therapy  
**GLAXOSMITHKLINE**



**David A H Whiteman**  
Global Clinical Development Lead  
**SHIRE PHARMACEUTICALS INC.**



**Chad Clark**  
President  
**PRECISION FOR MEDICINE**

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MED001654



#### FEATURED KEYNOTE

**MAKING  
PRECISION  
MEDICINE A  
REALITY IN OUR  
LIFETIME – THE  
FDA'S PLANS TO  
SUPPORT THE  
NEW PARADIGM**

**Dr. Robert Califf**  
FDA Commissioner

**“TO ME, [THE POWER OF] GENETICS AND GENOMICS TO HELP US UNDERSTAND THE MOLECULAR BASIS OF DISEASE IS CRUCIAL FOR BEING ABLE TO TARGET THERAPIES BETTER THAN WE COULD BEFORE.”**

**“BUT IT GOES BEYOND GENETICS. IF YOU LOOK AT THE PRECISION MEDICINE INITIATIVE, THE EMERGING USE OF WEARABLE TECHNOLOGY AND SOCIAL MEDIA ALLOWS US TO UNDERSTAND THINGS LIKE PATIENT PREFERENCES AND CONTINUOUSLY RECORD DATA THAT WE COULDN'T MONITOR BEFORE.”**

9:00	<b>PLENARY KEYNOTE: Precision medicine &amp; healthcare delivery: Making precision medicine a reality in our lifetime through technology</b> <ul style="list-style-type: none"><li>Unparalleled holistic and comprehensive approach to analyzing a patient's genomic and proteomic make-up</li><li>Precision treatment and clinical trials</li><li>Importance of connectivity at hospitals and medical centers in pushing precision medicine forward</li></ul> <b>Gary Palmer</b> , Chief Medical Officer, <b>NantHealth</b>			
9:20	<b>KEYNOTE PANEL: Putting a price on precision medicine: Is the dream of the Precision Medicine Initiative a financially feasible finale to the drug pricing wars?</b> <ul style="list-style-type: none"><li>Is linking drug pricing to performance a realistic solution to the ballooning costs of the interim personalized/genetic medicine approach of using post-prescription pharmacogenomics to determine individual drug efficacy?</li><li>Are payers prepared for a new reimbursement infrastructure, taking genomic diagnostics into account as we drive more towards a pre-emptive model?</li><li>Are current drug distribution models efficient and appropriate for future precision therapies, where efficacy and dosing will be pre-determined?</li></ul> <b>MODERATOR: Amupam B. Jena</b> , Faculty Research Fellow, National Bureau of Economic Research <b>Ed Pezalla</b> , Vice President, National Medical Director for Pharmaceutical Policy and Strategy, <b>Aetna</b> <b>David T. Ledbetter</b> , Chief Scientific Officer, <b>Geisinger Health System</b> <b>Steve Rosen</b> , Senior Director, Diagnostic Strategy, <b>Novartis</b>			
10:00	Networking Coffee Break and speed networking			
	<b>CELL THERAPY</b>	<b>GENE THERAPY</b>	<b>GENOMICS</b>	<b>PERSONALIZED HEALTHCARE</b>
11:00	Cell therapy and precision medicine: an overview <b>Robert Hariri</b> , Founder and CEO, <b>Celgene Cellular Therapeutics</b>	Gene therapy and precision medicine: an overview <b>Michael Linden</b> , Head of Gene Therapy, <b>Pfizer</b>	Genomics as the enabling force in precision medicine <b>Brad Perkins</b> , Chief Medical Officer, <b>Human Longevity Inc</b>	3D printing as an enabling technology in non-genomic precision medicine: what will it take to get there? <b>Jonathan Morris</b> , Assistant Professor of Neurology, <b>Mayo Clinic</b>
	<b>BIOPROCESSING AND MANUFACTURING</b>	<b>APPLYING GENOME ENGINEERING</b>	<b>CLINICAL IMPACT OF GENOMICS</b>	<b>NEWBORN AND PRENATAL DX</b>
11:20	Methods and technologies for analytical development: cell counting <b>Carl Simon</b> , Biologist, BioSystems and Biomaterials, <b>National Institutes of Standards and Technology</b>	TALEN Gene editing enabling “off the shelf” CAR-T <b>Andre Choulika</b> , Chairman and CEO, <b>Collectis</b>	Outcome prediction using a blood test – a pulmonary fibrosis case study <b>Naftali Kaminsky</b> , Professor of Medicine, <b>The Yale School of Medicine</b>	Rare disease diagnostics in newborns using whole genome sequencing <b>Benjamin Solomon</b> , Chief, Division of Medical Genomics, <b>Inova Healthcare Translational Medicine Institute</b>
11:40	Sourcing and qualification for precision cell therapies <b>Elizabeth Read</b> , Chief Scientific Officer, <b>Medeor</b>	Non-viral gene delivery as an alternative in the precision medicine paradigm <b>Rahul Aras</b> , CEO, <b>Juventas Therapeutics</b>	Personalized to precision – moving from anonymized data to actionable individual results for implementing pharmacogenomics markers in the clinic <b>Murray Brilliant</b> , Director, Center for Human Genetics, <b>Marshfield Clinic</b>	

	<b>BIOPROCESSING AND MANUFACTURING</b>	<b>APPLYING GENOME ENGINEERING</b>	<b>CLINICAL IMPACT OF GENOMICS</b>	<b>NEWBORN AND PRENATAL DX</b>
12:00	Cell standardisation strategies to ensure consistency in precision medicine development and delivery <b>David Brindley</b> , Director, <b>CASMI</b> (UK)	CRISPR gene editing technology for target development in Oncology <b>Jason Moffat</b> , Professor, <b>University of Toronto</b>	Practicing better clinical trials to achieve better medicine: targeting the 55% of patients with actionable variables pointing to a specific trial instead of a drug <b>Mark Gardner</b> , Chief Executive Officer, <b>OmniSeq</b>	Next generation counselling for prenatal precision medicine <b>Meghan Carey</b> , Executive Director, <b>National Society of Genetic Counselors</b>
12:20	Networking Lunch			
	<b>PLENARY ROUNDTABLES</b>			
2:00	<b>1</b> Working with patient organizations to develop cohorts and stratify patients <b>Andres Hourtado-Lorenzo</b> , Director of Translational Medicine, <b>Crohn's and Colitis Foundation</b> <b>Jak Knowles</b> , Managing Director, Vice President Medical and Scientific Affairs, <b>CureDuchenne Ventures</b> <b>Mark Rubin</b> , Director, Englander Institute for Precision Medicine, <b>Weill Cornell Medical Center</b> <b>Barbara Conley</b> , Associate Director, National Cancer Institute, <b>NIH</b>			
	<b>2</b> Prenatal diagnostics: capitalizing on whole genome sequencing rare disorders <b>Fred Lorey</b> , State Chief of Genetic Disease Screening Program (retired), Voting Member of Secretary's Advisory Committee on Heritable Disorders, <b>State of California</b> <b>Fred Miendl</b> , Sales Director North America, <b>Perkin Elmer</b> <b>David Whiteman</b> , Global Clinical Development Lead, <b>Shire</b>			
	<b>3</b> Genetic counselling: The expanding role in translational medicine <b>Erica Ramos</b> , Director-at-Large, <b>National Society of Genetic Counselors</b> <b>Jen Hoskovec</b> , Former President, <b>National Society of Genetic Counselors</b> <b>Katie Rock</b> , Genetic Counselor, <b>NIH</b> <b>Leila Jamal</b> , Genetic Counselor, <b>Baylor College of Medicine</b> <b>Toni Pollin</b> , Primary Investigator, <b>University of Maryland IGNITE Study</b> <b>Emily Edelman</b> , Genetic Counselor, <b>Jackson Labs</b>			
	<b>4</b> Informatics brain drain: maintaining the right talent at your firm for an evolving precision medicine industry <b>Mark Gardner</b> , Chief Executive Officer, <b>OmniSeq</b> <b>Robin Toft</b> , President and CEO, <b>Toft Group</b>			
	<b>5</b> Cell therapies: how to cope with an unsuccessful clinical launch <b>Kristin Comella</b> , Chief Scientific Officer, <b>US Stem Cell Inc.</b>			
	<b>6</b> From fastq to patient treatment guidance <b>Ying Chen</b> , Senior Bioinformatics Specialist in Oncology and Precision Medicine, <b>Cancer Institute of New Jersey</b> <b>Greg Volker</b> , Head of Oncology Bioinformatics, <b>GSK</b> <b>Amit Aggarwal</b> , Head of Bioinformatics, <b>Eli Lilly</b> <b>Yong Yue</b> , Head of Computational Biology, <b>Boehringer Ingelheim</b> <b>Bing Yuan</b> , Executive Director and Global Lead, Clinical Stage Oncology Business Development and Licensing, <b>Merck</b>			
	<b>7</b> NGS formats: their strengths and weaknesses as clinical tools <b>David Smith</b> , Professor of Laboratory Medicine and Pathology Chairman of the Technology Assessment Group Center for Individualized Medicine, <b>Mayo Clinic</b>			
	<b>8</b> Driving forward new pricing structures for precision medicine <b>Omar Ali</b> , Member of Adoption & Impact Programme Reference Panel, <b>NICE</b> (UK) <b>David Brindley</b> , Director, <b>CASMI</b> (UK)			

	COMMERCIALIZATION	ALTERNATIVE GENE THERAPIES	METABOLOMICS	COMPANION DIAGNOSTICS
2:40	Fund Raising to Partnering to Adoption and Commercial Success <b>Steven Deitcher</b> , Co-Founder and Board Member, <b>Medeor Therapeutics</b>	FCX-007 as a precision gene therapy for Recessive Dystrophic Epidermolysis Bullosa <b>John Maslowski</b> , SVP, Scientific Affairs, <b>Fibrocell Sciences</b>	Driving precision medicine forward through a joint metabolomics, genomics and microbiome analysis approach <b>Lavinia Ionita</b> , Chief Executive Officer, <b>Omixy</b>	Multiple in-vitro diagnostics for one biomarker: solving the problem <b>Rao Mulpuri</b> , Chief Operating Officer, <b>Provista Diagnostics</b>
3:00	The promise and realities of regenerative medicines <b>Marcie Glicksman</b> , Chief Scientific Officer, <b>ORIG3N</b>	Putting the patient first through AI analytics in combination therapy development <b>Niven Narain</b> , CEO, <b>Berg LLC</b>	Using metabolomics as a first-line phenotyping tool <b>Clary Clish</b> , Director of the Metabolomics Platform, Broad Institute, <b>MIT</b>	Unification of companion diagnostic development for speedier research <b>Prasun Mishra</b> , Precision Medicine Lead, <b>Genentech Roche</b>
3:20	Understanding the true value of cell therapy: how allogeneic therapies will drive the industry forward in a precision medicine world <b>Sicco Popma</b> , Scientific Director, Gene Modified Cell Therapy Leader, <b>Janssen</b>	Novel gene expression technologies as an alternative gene therapy driver in precision medicine <b>Francois Lebel</b> , Chief Medical Officer, <b>Ziopharm</b>	Patient selection approaches and actionable data using complex data from biomarker samples <b>Glenn Barnes</b> , Associate Director, Translational Medicine Operations, <b>Takeda Pharmaceuticals</b>	Using multiple sources of high-dimensional genomic data to build diagnostic algorithms <b>Anne-Marie Martin</b> , SVP, Head of Precision Medicine, <b>Novartis</b>
3:40	Networking Coffee Break			
	NOVEL TOOLS AND TECHNOLOGIES FOR REGENERATIVE THERAPIES	DATA ANALYSIS	COMPANION DIAGNOSTICS	
4:10	Extracellular Drug Conjugates: a small molecule/ antibody combination approach as an alternative to ADC technology in immunotherapy <b>James Prudent</b> , Chief Executive Officer, <b>Centrose Therapeutics</b>	Empowering limited data across communities: lessons from pediatric medicine <b>Adam Resnick</b> , Head of Precision Medicine Group, <b>Children's Hospital of Philadelphia</b>	The "strategic healthcare system": evaluating strategies for companion diagnostics in clinical development <b>Amir Handzel</b> , Statistical Science Director, <b>AstraZeneca</b>	
4:30	Cell and gene therapy delivery – utilizing iPSC-derived tissues as a simple solution for precision therapies <b>Eric David</b> , Chief Strategy Officer and EVP of Preclinical Development, <b>Organovo</b>	Cracking the code of copy number variants: generating a uniform set of exome data through ancestry decomposition <b>Jeffrey Reid</b> , Executive Director, Head of Genome Informatics, <b>Regeneron</b>	Physician-Driven assay design: decision support tools informed by leading practitioners of genetically guided patient care in real-world settings <b>Kristen Comella</b> , Chief Scientific Officer, <b>US Stem Cell Clinic</b>	PHYSICIAN-PHARMA INTERACTION

4:50	Combination therapy: Cell therapy as one piece in the puzzle to achieving precision medicine <b>Hans Klingemann</b> , Chief Scientific Officer, <b>NantKwest</b>	Landing among the stars: how "shooting for the moon" in translating genomic information to actionable results can end up providing valuable information – even when you miss <b>Kenna Mills Shaw</b> , Executive Director, Personalized Medicine Institute, <b>MD Anderson Cancer Center</b>
5:10	Enhancing autologous cell therapies for the precision era <b>Mike West</b> , CEO, <b>BioTime</b>	NCI-MATCH program: updates on the nation-wide trials and planning for the future <b>Barbara Conley</b> , Associate Director, National Cancer Institute, <b>NIH</b>
5:30	End of Conference and Networking Cocktails	



DAY 2

15<sup>th</sup> November, 2016

1/3

9:00

**PLENARY KEYNOTE: Precision medicine & healthcare delivery: Making precision medicine a reality in our lifetime through technology**

- Multi-sector partnerships to foster growth and innovation
- Building and regulating tools for detecting patients’ genomic patterns relevant to disease development, progression, and treatment
- Crowd-sourced, cloud-based informatics platform designed to advance the science and collaboration

**Dr. Robert Califf**, Commissioner, **US FDA**

**PLENARY KEYNOTE: Precision approvals: How biomarkers are blurring the lines in clinical trials and accelerating commercial launch**

- Biomarker driven patient stratification is bending the curve away from traditional Phase I, II and III studies– leading to accelerated approvals based on fewer patients, earlier
- Understanding the scientific, logistical and practical changes in design and execution of clinical trials for precision medicine
- Demonstrating value to payers and hospital systems and the healthcare system implications of every disease becoming a rare disease

**Chad Clark**, President, **Precision for Medicine**

9:30

Networking Coffee Break and speed networking

CELL + GENE THERAPY

IMMUNOTHERAPY

GENOMICS

GENOMIC IMPACT ON DISEASE RESEARCH

PERSONALIZED HEALTHCARE

ADVANCES IN HEALTHCARE IT FOR PRECISION MEDICINE

10:30

Engineered autologous T-Cell Therapy  
**Charles Nicolette**, CEO, **Argos Therapeutics**

Routine genomic research during drug development  
**Karina Bienfait**, Global Head, Genomic Strategy, Principal Scientist, **Merck**

Partnerships for co-development of an HIT infrastructure: advantages over in-house development  
**Shivdev Rao**, Director, Clinical Innovation Strategy, **UPMC**

11:00

Immunosequencing for a new class of immune diagnostics  
**Jianda Yuan**, Director, Translational Immuno-Oncology Research, **Merck**

Integrating natural histories into EHRs for more accurate diagnosis  
**David Pearce**, President of Research, **Sanford Health**

Enabling the integration of drug-related genetic findings into clinical practice  
**Peggy L. Peissig**, Ph.D., MBA, Chief Research Informatics Officer, **Marshfield Clinic**

11:30

Using small molecule epigenetic agents to induce biomimicry for immunotherapy and trigger tumor response  
**Stephen Baylin**, Deputy Director, The Sidney Kimmel Comprehensive Cancer Center, **Johns Hopkins**

**Cancer precision medicine:** What we know, what we think we know, and what we really need to know  
**Richard Buller**, Vice President, Oncology Clinical Development, **Pfizer**

Wellness as the cornerstone to precision medicine: wearable devices and data mining for early disease detection  
**Nathan Price**, Professor and Associate Director, **Institute for Systems Biology**

EPIGENETICS FOR CELL THERAPY + IMMUNOTHERAPY

CHANGING FACE OF KNOWN DISEASES

HEALTH DATA & ETHICS

1:30

Studying environmental contributions to human disease via epigenetics & genomics  
**Jan Scicinski**, SVP and Chief Scientific Officer, **EpicentRx**

**Pulmonary Disease** Precision medicine outside of oncology: developing the infrastructure for patient history-based studies  
**Claudia Rizzini**, Managing Director, Precision Medicine, **Brigham Women’s Hospital**

How can patient organizations generate valuable data – and use it to help push for precision cures for their disease?  
**Katherine Leon**, CEO, **SCAD Alliance**

DAY 2

15<sup>th</sup> November, 2016

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2:00

Novel Epigenetic therapies targeting angiogenesis, modifying metastasis by regulating epigenome  
**Kurtis Bachman**, Head of Computational and Systems Biology, **Janssen**

**PANEL: Rare Diseases** Is precision medicine the end of orphan drugs? How the orphan drug industry has impacted precision medicine  
**MODERATOR:** **Stephen Groft**, Former Director, Office of Rare Disease Research, **NIH**  
**PANELISTS:** **Yaffa Rubenstein**, Rare Disease Patient Registries Expert, **NIH**  
**Ed Pezalla**, Vice President, National Medical Director for Pharmaceutical Policy and Strategy, **Aetna**  
**Elizabeth Ottinger**, Project Manager, Rare Diseases, **NIH**  
**Abdel Halim**, Vice President, Translational Medicine, Biomarkers & Diagnostics, **Celldex Therapeutics**

Contributing genetic results to public databases for use in clinical development  
**Vanessa Rangel-Miller**, VP Genetic Services, **Patient Crossroads**

2:30

HEALTHCARE CIO-FOCUSED ROUNDTABLES

1

Genomic profiling moving into routine clinical care  
**Jeremy Segal**, Director, Division of Genomic Pathology, **University of Chicago**  
**David Roth**, Director, Penn Center for Precision Medicine, **University of Pennsylvania**  
**Kenna Mills Shaw**, Executive Director, Personalized Medicine Institute, **MD Anderson Cancer Center**  
**Steven Averbuch**, Vice President, Translational Clinical Development & Pharmacodiagnostics, **Bristol-Myers Squibb**  
**Abdel Halim**, Vice President, Translational Medicine, Biomarkers & Diagnostics, **Celldex Therapeutics**

2

Precision vs personalized: what's the difference in a clinical setting?  
**Peggy L. Peissig**, Chief Research Informatics Officer, **Marshfield Clinic**

3

Integrated ambulatory and hospital EHR systems: Small health system perspective  
**Daryl Kallevig**, Chief Information Officer, **Riverwood Healthcare**

DRUG DEVELOPMENT FOCUSED ROUNDTABLES

4

Robust HIT infrastructure: Challenges in development  
**Anup Kaneri**, Portfolio Strategist, **UPMC Enterprise**

5

Payer’s perspective: realities of reimbursement and precision medicine  
**Ed Pezalla**, Vice President, National Medical Director for Pharmaceutical Policy and Strategy, **Aetna**  
**Sam Johnson**, Director, Health Policy and Interprofessional Affairs, **American College of Clinical Pharmacy** (formerly of Kaiser Permanente)

6

Cost of goods sold in Cell and Gene therapies: overcoming low ROI through rare disease designation?  
**Elizabeth Ottinger**, Project Manager, Rare Diseases, **NIH**

7

Supply chain management for cell and gene therapies  
**Ena Bromley**, President and CSO, **BioStat Solutions**  
**Matt Nelson**, Director, Genetics, **GSK**  
**Michael Murray**, Director, Government Business Development, **Southern Research Institute**  
**Michelle Penny**, Director Computational Biology and Genomics, **Biogen**

8

Investing in precision medicine: Understanding what the capital markets are looking for in a disease-based investment world  
**Jak Knowles**, Managing Director, Vice President Medical and Scientific Affairs, **CureDuchenne Ventures**  
**Joshua Rsenick**, Partner Biotechnology, **SV Life Sciences**  
**Christina Bardon**, Managing Director, **MPM Oncology Impact Fund**  
**Christopher De Souza**, Director, **Broadview Ventures**  
**Jit Patel**, Director, **JDRF**

Networking Coffee Break

**DAY 2**

15<sup>th</sup> November, 2016

3/3

4:10

**PrecisionFDA SHOWCASE**

precisionFDA

This portion of the event is for companies who have been involved with the PrecisionFDA initiative to showcase their work to an industry audience through short, 10-minute segments, and highlight the findings of the PrecisionFDA competitions. Check back soon for more information!

5:10

End of Conference



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FEATURED KEYNOTE

**PRECISION MEDICINE &  
HEALTHCARE DELIVERY:  
MAKING PRECISION  
MEDICINE A REALITY IN  
OUR LIFETIME THROUGH  
TECHNOLOGY**

**Gary Palmer**  
CMO  
NANTHEALTH

NOVEL HOLISTIC AND COMPREHENSIVE APPROACHES  
TO ANALYZING A PATIENT'S GENOMIC AND PROTEOMIC  
MAKE-UP ARE STRESSING THE IMPORTANCE OF  
CONNECTIVITY AT HOSPITALS AND MEDICAL CENTERS  
IN PUSHING PRECISION MEDICINE FORWARD

MED001659

# WHAT IS PRECISION MEDICINE?

## ANSWER 1: A CATALYST FOR REGENERATIVE MEDICINE 2.0

A path to commercialization for Cell and Gene Therapies

## COMING FROM PHARMA & BIOTECH...



**Eric David**  
SVP Research and Development  
**ORGANOVO**

**TOPIC: Cell and gene therapy delivery – utilizing iPSC-derived tissues as a simple solution for precision therapies**

- Tech transfer in stem cells: achieving clinical-dose scale efficiently
- Helping to address the manufacturing gap using early preventative planning
- Understanding the regulatory framework for transitional technologies



**Michael Linden**  
Head of Gene Therapy  
**PFIZER**

**TOPIC: Gene therapy and precision medicine: an overview**

- Combining genomic disease knowledge and viral vectors for novel therapies
- AAV Gene therapy for hemophilia and blood disorders
- The issue of cost and ROI – can the precision medicine push overcome the pitfalls of Gene Therapy commercialization?



**Andre Choulika**  
Chairman and CEO  
**CELLECTIS**

**TOPIC: TALEN Gene editing enabling “off the shelf” CAR-T**

- Creating allogeneic CAR T-cells derived from healthy donors rather than the patients themselves
- Enabling development of product candidates that feature additional safety and efficacy attributes, including control properties designed to prevent them from attacking healthy tissues
- Enabling cells to tolerate standard oncology treatments, and equipping them to resist mechanisms that inhibit immune system activity



**Prasun Mishra**  
Precision Medicine Lead  
**GENENTECH ROCHE**

**TOPIC: Unification of companion diagnostic development for speedier research**

- Identifying drugs through HTS and lead identification using sequencing technologies
- Bridging diagnostic and therapeutic development during preclinical and clinical research
- Biomarker driven therapeutics – implementing metabolomics in the drug pipeline for disease identification.



**Carl Simon**  
**NATIONAL INSTITUTES OF STANDARDS AND TECHNOLOGY**

**TOPIC: Methods and technologies for analytical development: cell counting**

- Inability to reliably characterize cells as possibly industry's greatest challenge
- Issue addressed with systematic approaches for assessing sources of uncertainty and improving confidence in key measurements
- Applying these strategies will help to establish qualified assays for cell characterization which help streamline regulatory approval and enable more efficient development



**Amir Handzel**  
Head of Statistics  
**ASTRAZENECA**

**TOPIC: The “strategic healthcare system”: evaluating strategies for companion diagnostics in clinical development**

- Mass spectrometry for biomarker discovery in metabolomics – driving from translational to clinical developments
- Discussing the “cutoff” of companion diagnostics and regulatory incentives for the combined approach
- Developing a more strategic approach to clinical trial design using metabolomics and statistics



**Hans Klingeman**  
CSO  
**NANTKWEST**

**TOPIC: Combination therapy: Cell therapy as one piece in the puzzle to achieving precision medicine**

- NK Cells as a component in a larger precision medicine therapy paradigm
- Vaccine and antibody components, and how they interplay with cell therapy
- Using sequencing and proteomics to progress immunotherapy and checkpoint inhibitors



**Lavinia Ionita**  
CEO and Founder  
**OMIXY**

**TOPIC: Driving precision medicine forward through a joint metabolomics, genomics and microbiome analysis approach**

- Cross-analyzing multi-omics testing results, to make sure that all of the pieces are being seen
- Showing the biochemical changes that occur in the organism during metabolic processes, to further understand physiology
- Understanding ageing physiology to extend lifespan



**Steven Deitcher**  
**BIOTECHNOLOGY INVESTOR**

**TOPIC: Fund Raising to Partnering to Adoption and Commercial Success**

- Driving commercial viability of autologous cell therapy through new matrices
- Immunotherapy and combination therapies through accelerated FDA tracks



**Richard Buller**  
Vice President and Head, Clinical Development  
**PFIZER ONCOLOGY**

**TOPIC: Cancer precision medicine - What we know, what we think we know, and what we really need to know**

- Insights into patient selection strategies
- What is the testing implementation on the path to approval and the highway post-approval?
- Best practices when partnering with health authorities
- What are some of the issues for resolution in the cost model?



**Robert Hariri**  
Founder and CEO  
**CELGENE CELLULAR THERAPEUTIC**

**TOPIC: Cell therapy and precision medicine: an overview**

- How autologous cell therapies have laid the groundwork for precision medicine
- Moving forward – working towards manufacturing to scale and standardization
- What the coming new drug pricing infrastructure means for cell therapy producers



**Rahul Aras**  
CEO  
**JUVENTAS THERAPEUTICS**

**TOPIC: Non-viral gene delivery as an alternative in the precision medicine paradigm**

- Utilizing endogenous stem cell repair as a therapeutic function
- Preventing inflammation and immunogenicity in a viral vector
- Enabling treatment in cardiac, organ and skin tissue damage through low-intrusion therapies



**Karina Bienfait**  
Global Head, Genomic Strategy, Principal Scientist  
**MERCK**

**TOPIC: Routine genomic research during drug development**

- Informing the development strategy using genomic data acquired from clinical trials
- Development of set goals and distinct policy in relation to obtaining genomic information during PhI/II/III clinical trials.
- Working with patient groups and using natural history research as a catalyst for routine testing



**Mike West**  
CEO  
**BIOTIME INC. and Board Member LIFEMAP SOLUTIONS**

**TOPIC: Enhancing autologous cell therapies for the precision era**

- Driving commercial viability of autologous cell therapy through new matrices
- Immunotherapy and combination therapies through accelerated FDA tracks



**Jeffrey Reid**  
Executive Director, Head of Genome Informatics  
**REGENERON**

**TOPIC: Cracking the code of copy number variants: generating a uniform set of exome data through ancestry decomposition**

- Growing a uniform set of exome data through determining associations in genotypes and phenotypes
- 85,000 exomes from sequence and lab data to build a genomic and metabolomics infrastructure
- Identifying co-normalization through k means clustering



**Kurtis Bachman**  
Head of Computational and Systems Biology  
**JANSSEN**

**TOPIC: Novel Epigenetic therapies targeting angiogenesis, modifying metastasis by regulating epigenome**

- Epigenetics can answer the question of stem cell efficacy and lineage changes
- Epigenetic memory and retained programming as both a marker and tool
- Cancer stem cell hypothesis and epigenetic biomarkers in oncology



**John Maslowski**  
SVP, Scientific Affairs  
**FIBROCELL SCIENCES**

**TOPIC: FCX-007 as a precision gene therapy for Recessive Dystrophic Epidermolysis Bullosa**

- Addressing the underlying cause of RDEB by providing functional Type VII collagen to affected areas
- Fibroblast genetically modified to express functional COL7
- Dermal fibroblasts are collected from the patient, autologous therapy



**Elizabeth Read**  
Chief Scientific Officer  
**MEDEOR**

**TOPIC: Sourcing and qualification for precision cell therapies**

- Cellular starting materials and approaches for their sourcing
- Specific challenges in starting material qualification for patient-specific products
- Off-the-shelf allogeneic products (multipotent or pluripotent stem cell-derived) for patient unspecific treatments



**Niven Narain**  
CEO  
**BERG LLC**

**TOPIC: Putting the patient first through AI analytics in combination therapy development**

- Using deep learning AI to understand patient needs at a genomic and phenotypical level
- Developing a global architecture to connect the dots and give us a picture of the activity of the biological systems within the patient
- Real time analytic solutions for predicting the impact of treatment plans at the individual level to optimize population health strategies



**Jan Scicinski**  
SVP and Chief Scientific Officer  
**EPICENTRX**

**TOPIC: Restoring efficacy of immunotherapy treatments in oncology with radical oxygen and nitrogen based epigenetic drugs**

- Acquired resistance to chemotherapies results in early disease progression and lower survival
- ROS-mediated episensitizers hold the promise of not only restoring efficacy to immunotherapy treatments
- RfRx-001-mediated resensitization in the context of an ongoing Phase 2 clinical trial in metastatic colorectal cancer



**James Prudent**  
President and CEO  
**CENTROSE THERAPEUTICS**

**TOPIC: Extracellular Drug Conjugates: a small molecule/antibody combination approach as an alternative to ADC technology in immunotherapy**

- Using well-conserved sodium protein pump overexpression in cancer cells to our advantage
- Targeting inhibitor of pump in B-cell lymphomas
- Unique approaches to using the small molecule approach to drive precision medicine forward

# WHAT IS PRECISION MEDICINE?

## ANSWER 2: PUTTING THE PATIENT FIRST

Pharma and the top provider systems in the world will be showcasing how they are driving forward expanding treatment methods and growing their HIT infrastructure to match the growing genomics data boom.

## COMING FROM HEALTHCARE PROVIDERS & PATIENT ORGANIZATIONS...



**Jonathan Morris**  
Assistant Professor of Neurology  
**MAYO CLINIC**

**TOPIC: 3D printing as an enabling technology in non-genomic precision medicine: what will it take to get there?**

- Perfecting imaging – how we can develop protocols for 3D printing and enhance segmentation tools towards patient specific tumors and wounds
- How to improve software through intrinsic anatomic knowledge
- Faster, more reliable 3D printers using better materials as a catalyst for precision healthcare



**Adam Resnick**  
Head of Precision Medicine Group  
**CHILDREN'S HOSPITAL OF PHILADELPHIA**

**TOPIC: Empowering limited data across communities: lessons from pediatric medicine**

- Building an infrastructure around specimens and special patient communities
- Integration between stakeholders in consumer and commercial space
- Standardization of SOPs and organization of data availability



**Murray Brilliant**  
Director  
**MARSHFIELD CLINIC RESEARCH FOUNDATION**

**TOPIC: Personalized to precision – moving from anonymized data to actionable individual results for implementing pharmacogenomics markers in the clinic**

- Identifying “actionable genes” to reduce contra-indicated drug assignments
- Placing pharmacogenomics markers within EHRs to prevent interference with the clinical workflow
- Reducing the hindrance to preventative care of patients changing healthcare providers using HER interoperability



**David Pearce**  
President of Research  
**SANFORD HEALTH**

**TOPIC: Integrating natural histories into EHRs for more accurate diagnosis**

- Implementation of newborn screening at the state level to develop natural histories
- Importance of interoperability for EHR usage in diagnosis
- Impact of natural history screenings for Batten disease through gene therapy clinical studies



**Nathan Price**  
Professor and Associate Director  
**INSTITUTE FOR SYSTEMS BIOLOGY**

**TOPIC: Wellness as the cornerstone to precision medicine: wearable devices and data mining for early disease detection**

- Understanding the interface between genome sequencing and wearable devices for individual patient wellness
- Identification of actionable genes through data mining for early diseases
- Using the patient-physician relationship as a vehicle for delivery of wellness information



**Naftali Kaminski**  
**BOEHRINGER INGELHEIM PHARMACEUTICALS, INC.**  
Professor of Medicine  
**YALE UNIVERSITY SCHOOL OF MEDICINE**

**TOPIC: Outcome prediction using a blood test – a pulmonary fibrosis case study**

- Solving the issue of early transplantation through utilization of modeling effects on referrals.
- Distinguishing patient drug response through genotyping and metabolic identification
- Precision medicine isn't just genetics - Identifying innate immunity markers for biomarker development and target discovery



**Clary Clish**  
Head of Genomics  
**BROAD INSTITUTE**

**TOPIC: Using metabolomics as a first-line phenotyping tool**

- Population-based studies for metabolomics signs of disease
- Analyzing population-based cohorts for large-scale studies and developing model systems
- Monitoring biomarkers that can change the course of individual treatments



**Andres Hourtado-Lorenzo**  
Director of Translational Medicine  
**CROHN'S AND COLITIS FOUNDATION**

**ROUNDTABLE DISCUSSION: Working with patient organizations to develop cohorts and stratify patients**



**Stephen Baylin**  
Deputy Director  
**THE SIDNEY KIMMEL COMPREHENSIVE CANCER CENTER, JOHNS HOPKINS**

**TOPIC: Using small molecule epigenetic agents to induce biomimicry for immunotherapy and trigger tumor response**

- Clinical data - triggering interferon response and immune response to begin tumor destruction
- DNA demethylation agents and acetylation inhibitors as genome-altering agents for oncology
- Understanding the abnormalities of chromatin and methylation assembly that may account for the appearance of epigenetic abnormalities during tumor development, and how they mediate the transcriptional repression



**Claudia Rizzini**  
Managing Director, Precision Medicine  
**BRIGHAM WOMEN'S HOSPITAL**

**TOPIC: Pulmonary Disease – Precision medicine outside of oncology: developing the infrastructure for patient history-based studies**

- Genomic sequencing is becoming faster, cheaper, more accurate, and more available in a variety of contexts.
- One major use-case for genomic sequencing is the ability to diagnose rare diseases (which are common in aggregate), short-circuiting long, difficult, and expensive “diagnostic odysseys.”
- In addition to providing answers, diagnosis through genomics may also impact medical care for patients both immediately and in the longer-term.



**Benjamin Solomon**  
Chief, Division of Medical Genomics  
**INOVA HEALTHCARE TRANSLATIONAL MEDICINE INSTITUTE**

**TOPIC: Rare disease diagnostics in newborns using whole genome sequencing**

- Coupling diagnostics with genetic counseling
- Tests to evaluate the health of both mother and baby even during the first trimester
- High resolution 4-D transvaginal probe that helps physicians view and detect fetal abnormalities earlier than ever



**Katherine Leon**  
President  
**SCAD ALLIANCE**

**TOPIC: How can patient organizations generate valuable data – and use it to help push for precision cures for their disease?**

- Supporting patients and caregivers while educating clinicians on the role that patient groups can undertake
- Identification of genetic risk factors for a rare, seemingly random complication from disease and surgery
- Developing a registry for SCAD patients and family members, including genomic data



**Jeremy Segal**  
Director, Division of Genomic Pathology  
**UNIVERSITY OF CHICAGO**

**ROUNDTABLE DISCUSSION: Genomic profiling moving into routine clinical care.**



**Brad Perkins**  
Chief Medical Officer  
**HUMAN LONGEVITY INC.**

**TOPIC: Genomics as the enabling force in precision medicine – attaching meaning to the sequence**

- How machine learning capabilities are transforming the precision medicine landscape
- Predicting photograph-quality images from the genome
- Attaching real meaning to the genome sequence through unique bioinformatics platforms integrating pharma, healthcare and reimbursement



**Patricia Weltin**  
CEO  
**RARE DISEASE UNITED FOUNDATION**

**TOPIC: Putting the patient first – the vision of Precision Medicine from the Patient Perspective**

- Understanding the driving forces behind rare disease research as the precursor behind precision medicine
- Emphasizing the role of patient groups and natural histories as a catalyst for precision therapies
- What is the future for rare diseases, and rare disease patient groups? How will they play into the new paradigm?



**Daryl Kallevig**  
Chief Information Officer  
**RIVERWOOD HEALTHCARE**

**ROUNDTABLE HOST: Integrated ambulatory and hospital EHR systems: Small health system perspective**



**Rasu Shretha**  
Chief Information Officer  
**UPMC**

**TOPIC: Partnerships for co-development of an HIT infrastructure: advantages over in-house development**



**Neal Ganguly**  
Chief Information Officer  
**JFK HEALTH SYSTEM**

**TOPIC: Investing wisely in the right tools to entice patients to engage with healthcare providers**

# PARTICIPATING ORGANIZATIONS



# REASONS TO ATTEND

- 1 Hear from **FDA Commissioner Robert Califf** in a historical keynote on Precision Medicine and the FDA's future plans to help drive the industry.
- 2 Join the most comprehensive agenda on commercial and scientific challenges in **Cell and Gene Therapies**, as outlined from pharmaceutical giants like **Celgene** and **Merck**, as we drive next-generation therapeutics towards the new precision paradigm.
- 3 See brand new prenatal and newborn diagnostics technology being implemented in healthcare clinics, such as **Inova Health Systems**, and how early genomic testing will change how we do medicine.
- 4 Learn how **pharma** and **healthcare providers** are **implementing technological advances** into their clinical and research workflow from the CIOs of **UPMC**, **Riverwood Healthcare** and **JFK Health System**.
- 5 **Where big pharma meets big healthcare:** Be a part of the largest gathering in North America of pharma and providers towards moving precision medicine forward, **bridging the gap between genomic information and R&D**.
- 6 **Witness** groundbreaking high-level talks on the implementation of the PMI in the private sector, from speakers like **Prasun Mishra**, Precision Medicine Lead at **Genentech Roche** and **Jeff Reid**, Director of Genomics, **Regeneron**.
- 7 **Sit down** with **Jeremy Segal**, Director of Genomics at **University of Chicago**, as he discusses moving **genomic profiling** into routine clinical care.
- 8 **Rare Diseases:** See how the evolving precision medicine paradigm is changing the face of the Orphan Drug industry and rare diseases, from the perspective of **payers, patients and pharma**.
- 9 **Putting the patient first:** A wide array of perspectives from patient groups actively compiling natural histories of their diseases, including the **Crohn's and Colitis Foundation**, and the **Rare Disease United Foundation**.
- 10 Meet, discuss research, and do business with hundreds of other industry leaders using the **Jublia Networking System**, where you can search not just by name and title, but by the content of their work.

Register now at [www.terrapinn.com/attendprecision](http://www.terrapinn.com/attendprecision)

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DELEGATE BOOKING			
PACKAGE	BEFORE Oct 7 <sup>th</sup>	BEFORE Oct 28 <sup>th</sup>	FINAL PRICE
2 day conference	\$2,250 <b>SAVE \$225</b>	\$2,365 <b>SAVE \$110</b>	\$2,475
Group of 3	\$1,690	\$1,775	\$1,855
Group of 6+	\$1,465	\$1,535	\$1,610

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## SEEKING GLOBAL SYNERGY BETWEEN BUSINESS & SCIENCE

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### 2019 Linus-Pauling Speakers (to be announced) and our past Linus Pauling biotech pharma Symposium speakers (see jpg below)

 Thomas Waldmann MD NIH/NCI <b>1<sup>st</sup> Keynote</b>	 Prof Scott Mohr BU	 Martine Darwish Genentech	 James Ernst PhD Genentech	 Kate Senger PhD Genentech	 Shannon Chilewski Bristol Myers Squibb	 Dr. Andreas Schaaf CSO, Greenovation Germany
 Andrew Bradbury MD PhD Los Alamos National Lab <b>2<sup>nd</sup> Keynote</b>	<b>Linus-Pauling Biologics/Antibody Symposium</b> April 24, 2016, Boston, Seminar fee \$ 0 <a href="http://www.Nobel-Pauling.org">www.Nobel-Pauling.org</a>					 Dr. Michael Blank CSO, AptaiT Germany
 Paul Carter PhD Genentech <b>3<sup>rd</sup> Keynote</b>	 <b>1954 Nobel Prize in Chemistry</b> <b>1962 Nobel Peace Prize</b> <a href="http://www.studentvision.org">www.studentvision.org</a>					 Dr. Stefan Dübel Founder, YuMab Germany
 Jeffrey Siegel MD Genentech	 Randall Brezski PhD, Genentech	 Carolyn Cuff PhD AbbVie	 Vibha Jawa PhD Amgen	 Dr. Stefan Weigand Roche Switzerland	 Grace Wong PhD CSO, ActoKine Therapeutics	

Pre-registration is required but seminar fee is \$0.

Themes: Biologics, Antibody Therapeutics and Immunotherapy

What is hot in biotech pharma? Why mentors?

Why science? Why open innovation and global collaboration?

Themes - Career decisions: academia vs. biotech pharma for science discovery

Yin & Yang: lessons learned from working in academia, biotech & pharma.

If innovation is a seed, where is the soil? When will it rain?

2016 Nobel-Pauling Biologics/Antibody Symposium Agenda

**Agenda**

2:00 pm	Registration, networking and group photo with speakers
3:00-5:00 pm	<p>Welcome: Dr. Grace Wong, ActoKine Therapeutics (former Genentech) Legacy of Dr. Linus Pauling: Professor Scott Mohr, Professor of Chemistry at Boston University</p> <p>Presentation: Biologics, Antibody Therapeutics and Immunotherapy Moderators: Dr. Ravi Kumar, CSO, Acceleron Pharma, Dr. Joseph Murphy, CRL &amp; Dr. Jeff Boucher, UMass Medical School</p> <p>Dr. Thomas Waldmann, MD, Head, Cytokine Immunology and Immunotherapy Section, National Cancer Institute (Keynote) Seminar title "IL-2 and IL-15 in the life and death of lymphocytes: Serendipity and a 60 year scientific odyssey"</p> <p>Dr. Andreas Schaaf PhD, Greenovation Biotech, Germany Martine Darwish, MS, Senior Scientist, Protein Chemistry, Genentech Dr. James Ernst, PhD, Senior Scientist, gRED Research and Early Development, Genentech</p> <p>Prof. Dr. Stefan Dübel PhD, Technische Universität Braunschweig, Germany (co-founder of YuMab GmbH) Seminar title: Human therapeutic antibodies and beyond + career advice for students, postdocs &amp; scientists</p> <p>Dr. Michael Blank PhD, CSO, AptaIT GmbH, Munich, Germany Shannon Chilewski, MS, Scientist, Bristol-Myers Squibb</p>
5:00-6:00 pm	<p>Panel discussion: Why Biologics, Antibody Therapeutics and Immunotherapy? Career decision: academia vs. industry (all speakers)</p>
6:00-6:10 pm	<p>Dr. Grace Wong: New drug discovery: AK-2 for protection against infection by a broad spectrum of viruses Dim Sum and group photo with speakers, students, postdocs and scientists</p>
7:00-7:40 pm	<p>Presentation: Biologics, Antibody Therapeutics and Immunotherapy Moderators: Dr. Ibraheem Badejo, Johnson &amp; Johnson, Dr. Pallab Ghosh, Harvard Dr. Ward Yuhas, CRL &amp; Dr. Qing Liao (Boston Paragon and Spire Metering Technology)</p> <p>Dr. Andrew Bradbury, MD, PhD, Staff Scientist, Biosciences, Los Alamos National Laboratory (Keynote) Dr. Randall Brezski, PhD, Scientist, Antibody Engineering, Genentech Dr. Carolyn Cuff, PhD, Leader, Translational Research &amp; Investigation Unit, AbbVie</p>
7:40-9:30 pm	<p>Dr. Paul Carter, PhD, Senior Director and Staff Scientist, Antibody Engineering, Genentech (Keynote) Seminar title - Antibody Therapeutics: Past, Present and Future (+ career advice)</p> <p>Jeffrey Siegel, MD, Senior Group Medical Director, Global Head, Genentech Seminar title: Biologics for Autoimmune diseases? + career advice for students, postdocs &amp; scientists</p> <p>Dr. Vibha Jawa, PhD, Principal Scientist, Clinical Immunology, Amgen Dr. Stefan Weigand PhD, Head of Large Molecule Research, Roche</p> <p>Panel discussion: Why Biologics, Antibody Therapeutics and Immunotherapy? Career decision: academia vs. industry (all speakers)</p> <p>Closing remark: Dr. Grace Wong (former Genentech) ActoKine Therapeutics</p>
9:30-10:00 pm	Networking for skills & advice (sea of learning)

2015 Nobel-Pauling Biotech Pharma Christmas Symposium agenda and speakers



Let Nobel-Pauling's candle light up the path to new drug discovery  
[www.Nobel-Pauling.org](http://www.Nobel-Pauling.org)

**2015 Nobel-Pauling Biotech Pharma Christmas Symposium speakers**

A moment lasts all of a second, but the memory lives on forever









Dr. Linus Pauling

2015. 12. 25

It is not what's under the Christmas tree that matters, it's who around it

### Agenda

2:00 pm	Registration, networking with Dim Sum and group photo with speakers
3:30-4:30 pm	<p>Welcome: Dr. Grace Wong, ActoKine Therapeutics  Legacy of Dr. Linus Pauling: Amadou Barry, South Africa, UMD</p> <p>Presentation: the past, the present &amp; the future &amp; Why career in Science  Moderators: Dr. Xin Tang, MIT &amp; Dilip G. Gosar (former Lilly)  Dr. Jiang He, MIT  Dr. Christian Maass, MIT, from Germany  Dr. Yogesh Dayma, MIT  Dr. Ramchander Chepyala, MIT  Dr. Tianmin Fu (Prof Hao Wu's lab), Harvard</p>
4:30-5:00 pm	<p>Smart pitch  Dr. Xin Tang (Harvard); Dilip Gosar (former Lilly); Dr. Rajiv Shrestha (Octet Research);  Yogesh Reddy (Ascensus); Jessica Val (UMD); Alvin Lu (Harvard); Dr. Zhao Wang (MIT);  Dr. Zhou Lin (MIT); Dr. Peng Du (Tufts); Weiming Sun (MIT Sloan); Dr. Jicong Cao (MIT);  Dr. Jung Suh (MIT); Dr. Ahmed Fazly (MIT); Dr. Claudia Wehrspaun (MIT);  Dr. Jun Yang (MIT); Dr. Ru Wang (MIT)</p>
5:00-5:20 pm	<p>Dr. Grace: What are the most important criteria for success in scientific discovery?  Getting a foot in the door &amp; What is hot in Biotech Pharma?</p>
5:30-6:30 pm	<p>Yin &amp; Yang: lessons learned from working in academia, biotech &amp; pharma.  Moderators: Dr. Rajiv Shrestha (Octet Research Inc) &amp; Yogesh Reddy, Ascensus (Investment)  Dr. Yue Liu, Pfizer  Dr. Robert Ng, BioMarin Pharmaceuticals  Supriya Shekar, Medbiomarkers  Xiaoyue Shi, Case Western Reserve University, Ohio (Why China Biotech Pharma?)</p>

Prof DAI Huanqin is an associate professor in Prof Gil Alterovitz's lab (MIT/Harvard), as a visiting scholar from the Chinese Academy of Sciences (in Prof Lixin Zhang's lab), Beijing, China

6:30-7:00 pm


Dr. Grace (Career decisions: academia vs. Industry (start up, biotech & pharma)  
Closing remark: Dr. Grace (founder of [www.ActoKine.com](http://www.ActoKine.com); [www.Nobel-Pauling.org](http://www.Nobel-Pauling.org) and [www.StudentVision.org](http://www.StudentVision.org))

7:00-8:00 pm


Networking for skills & advice (sea of learning)

## 2Nobel-Linus-Pauling Biotech Pharma **microRNA** Symposium

[www.Nobel-Pauling.org](http://www.Nobel-Pauling.org)




Dr. Linus Pauling



[www.Nobel-Pauling.org](http://www.Nobel-Pauling.org)


Prof Roy Glauber  
2005 Nobel Laureate  
Harvard



Prof Arthur Pardee  
Father of  
Dana-Farber  
Cancer Institute  
Harvard

March 17 2014  
Fee \$ 0

A moment lasts all of a second, but the memory lives on forever



Dr. Muller  
Fabbri  
USC, LA

Dr. Rounak  
Nassirpour  
Pfizer

Dr. David Salzman  
Biogenidec

Prof Arthur Pardee  
Pauling student  
Harvard

Prof Roy Glauber  
(Nobel 2005)  
Harvard

Dr. Grace Wong  
Nobel-Pauling  
Studentvision.org

Dr. Prasun  
Mishra  
NIH

Prof Lorenzo  
Sempere  
VA Res. Inst

### 2014 Nobel-Pauling microRNA and mentor Symposium Agenda

Boston (this location is shown only to confirmed attendees)

2014 Nobel Linus Pauling Mentoring & MicroRNA Symposium (March 17, 2014)

#### Agenda

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3:00 pm

Registration & Networking with substances

3:30 pm






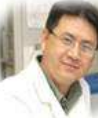




















Welcome: Dr. Grace Wong, CEO, ActoKine Therapeutics  
Legacy of Dr. Linus Pauling: Marsha Paul, Linus Pauling Volunteer, volunteer for StudentVision.org  
Dr. Chung-Wei Lee, MIT & Dr. Weimin Guo, Harvard  
Bryan Germain, BiogenIdec, Career Keynote: Why career in Biotech?  
Dr. David Salzman, BiogenIdec, Educational Keynote: microRNA: past, present & future

4:30-5:00pm

Speakers:  
Smart pitch from speakers from academia, biotech & pharma  
Dr. Chung-Wei Lee, MIT; Dr. Weimin Guo, Harvard;  
Dr. David Salzman, BiogenIdec; Dennis France, Advanced Cellular Dynamics  
Dr Grace Wong, ActoKine Therapeutics (1st seminar Title: AK-1 for cancer and AK-2 for bird flu prevention)  
2nd seminar title: Advice from Nobel Laureates and experts: What are the most important criteria for success

	in scientific discovery? and other speakers (students, post-docs & speakers from Academia, biotech & pharma). Prof Arthur Pardee, Harvard; Dr. James Falls, GSK; Dr. Xuemei Zhao, Merck; Dr. Rounak Nassirpour, Pfizer; Dr. Pavan Kumar, Eisai; Dr. Prasun Mishra; NIH; Prof Muller Fabbri; USC, LA: Prof Lorenzo Sempere, Van Andel Res. Inst; Prof Robert Lee, OSU, Ohio; Prof Taosheng Chen, stjude.org; Prof John Pezacki, Univ of Ottawa; Dr. Kathy Martin, KJMBiosystems; Becky Buzzeo, ThermoFisher Scientific; Dr. Jingfang Ju, Stony Brook Univ and more.
5:30 pm	Speaker group photo with students & postdocs at 5:40pm and surprise pitch from Prof Pardee's four postdocs at 5:50pm Prof Charles Stiles, Harvard (surprise pitch for Prof Pardee) Keynote speakers: 6:00pm Prof Arthur Pardee, Harvard (Linus Pauling's student at Caltech) Title: microRNAs and therapy of cancer Prof Roy Glauber, Nobel Laureate, Harvard (story of Linus Pauling)
6:30-7:30 pm	Speakers from Academia and biotech pharma Industry: microRNA seminars? Moderators: Dr. David Salzman, BiogenIdec & Alex Pauling, Nobel-Pauling Prof Arthur Pardee, Harvard; Dr. Bing Xia, GSK; Dr. Xuemei Zhao, Merck Dr. James Falls, GSK; Dr. Rounak Nassirpour, Pfizer; Dr. Pavan Kumar, Eisai; Dr. Prasun Mishra; NIH; Prof Muller Fabbri; USC, LA: Prof Lorenzo Sempere, Van Andel Res. Inst; Prof Robert Lee, OSU, Ohio; Prof Taosheng Chen, stjude.org; Prof John Pezacki, Univ of Ottawa and more.....
7:30 pm - 8:00 pm	Biotech who's who (the joy of learning)
8pm-9pm	Panel discussion: Why microRNA? Prof Arthur Pardee, Harvard; Dr. James Falls, GSK; Dr. Xuemei Zhao, Merck; Dr. Rounak Nassirpour, Pfizer; Dr. Pavan Kumar, Eisai; Dr. Prasun Mishra; NIH; Prof Muller Fabbri; USC, LA: Prof Lorenzo Sempere, Van Andel Res. Inst; Prof Robert Lee, OSU, Ohio; Prof Taosheng Chen, stjude.org; Prof John Pezacki, Univ of Ottawa; Dr. Kathy Martin, KJMBiosystems; Becky Buzzeo, ThermoFisher Scientific; Dr. Jingfang Ju, Stony Brook Univ and more....
9:00 pm	Closing remark (Dr. Grace Wong, founder of Nobel-Pauling.org and StudentVision.org)
9:10-10pm	Networking for skills & advice (sea of learning to be useful for the world)

2014 Nobel-Linus-Pauling Biotech Pharma Symposium, [www.Nobel-Pauling.org](http://www.Nobel-Pauling.org), seminar fee \$ 0

 Dr. Rounak Nassirpour Pfizer	 Prof Robert Lee OSU, Ohio	 Dr. James Falls GSK	 Dr. Prasun Mishra NIH	 Dr. Pavan Kumar Eisai	 Dr. Jingfang Ju Stony Brook Uni	 Prof Lorenzo Sempere Van Andel R I
 Dr. David Salzman Biogenidec	 Dr. Chiang Li Pardee's postdoc BostonBioMedical	<b>Nobel-Pauling microRNA Symposium</b> March 17, 2014, Boston <a href="http://www.Nobel-Pauling.org">www.Nobel-Pauling.org</a>  1954 Nobel Chemistry 1962 Nobel Peace  <a href="http://www.Nobel-Pauling.org">www.Nobel-Pauling.org</a>			 Dr. Xuemei Zhao Merck	 Prof Taosheng Chen Stjude.org
 Dr. Muller Fabbri USC, LA	 Prof Charles Stiles Harvard	 Prof Arthur Pardee Pauling student	 Prof Roy Glauber (Nobel 2005)	 Bryan Germain Biogenidec	 Dr. John Pezacki Univ of Ottawa	
 Dr. Weimin Guo Harvard	 Dr. Kathy Martin Pardee's postdoc KJMBiosystems	 Dr. Lili Huang Pardee's postdoc Abbvie	 Dr. Chung-Wei Lee MIT	 Marsha Paul Volunteer	 Becky Buzzee ThermoFisher	 Dr. Grace Wong ActoKine Therapeutics

Education should be free like air. Science brings people together.

## 2013 Nobel-Pauling Biotech Pharma Symposium Agenda

Boston (this location is shown only to confirmed attendees. Please call 617-566-0511 for the location)  
2013 Christmas (11 am to 5 pm)

### Agenda

11:00 am	Registration & Bio who's who (networking, 12 to 3pm)
12:00 pm	Welcome: Dr. Grace Wong, ActoKine Therapeutics Legacy of Dr. Linus Pauling (Dr. Jeremy Kintigh, University of New Hampshire)
12:30 pm	Keynote speakers (New drug discovery: the need for innovation & global collaboration) Dr. Michael Lam, Merck; Dr. Nanding Zhao, Eisai; Dr. Xiaoyu Tian, Bristol-Myers-Squibb; Dr. Jeff Ding, Sanofi; Dr. Bob Ward, AstraZeneca; Dr. Sherwin Shang, Abbvie; Dr. Yufei Xu, Novartis  Speakers from Academia and Industry for science innovation Moderators: Richard Shamon, Student Vision & Alex Pauling, Nobel-Pauling Sean Yang, genzyme; Yu Zhang, Pfizer; Dr. Emile Bellott, Howfong, China; Dr. CN Ramchand, CEO, Laila Pharma, India; Dr. Grace Wong, ActoKine Therapeutics; Dr. Min Wu, Harvard; Dr. Yansheng Hao, DFCI/Harvard; Dr. Haiyan Peng, Biogenidec; Dr. Shenghong Yang, Harvard and Dr. Lichao Chen, Harvard; Dr. Leonid Gaidukov, MIT; Dr. Nikola Kojic, MIT; Prof Maolin Guo, UMass Dartmouth; Dr. Alper Kucukural, UMass Medical School; Dr. SHANKAR PRASAD DAS, UMass Medical School
2:00 pm	Smart pitch – Bio Who's who (the joy of learning)
2:15 pm	Short presentation: Why scientist? The past, the present & the future. Dr. Leonid Gaydukov, MIT; Dr. Shenghong Yang, Harvard; Dr. Aishuang Xiang, Novartis-MIT; Dr. Alper Kucukural, UMass Medical School; Amanuel Ghidry, University of New Hampshire;

Dr. Nikola Kojic, MIT & Dr. Zina Zhu, MIT; Dr. SHANKAR PRASAD DAS, Umass Medical School; Dr. Ahmed Bayoumi MD, Ibgem; Adil Malam, Harvard, UK

#### Smart pitch-Biotech who's who

Dr. Ozlem YILDIRIM, Harvard; Dr. David Dai, Merck; Dr. Yuqi Qin, Dr. Haiyan Peng, BiogenIec Dr. Sha Mi, BiogenIdec; Dr. Liu shubai, Harvard and Hua Wang, Northwestern University, Chicago; Dr. Saibal Chakraborty, NIH; Dr. Ming BAI, Harvard; Dr. Helen Sadik, Johns Hopkins Medical School & Michel Lau, Johns Hopkins U; Dr. Cong Peng, Harvard; Dr. Chung-Jung Chiu, Tufts University; James Martin II, Princeton University; Dr. Shubai Liu, Harvard; Dr. Liling Zeng, BU; Shi Su in Dr. Tai Chen's lab, Boston University; Prof Smita Varia, Dr. Gunasekaran Singaravelu & Jonathon Brzezinski, Rutgers University; Dr. Minh Le, from Prof. Harvey Lodish's lab, Boston Children's Hospital; Dr. Gillian Browne, University of Vermont College of Medicine; Dr. Jiahai Shi, from Prof. Harvey Lodish's lab, Whitehead Institute for Biomedical Research; Dr. Xiaohua Huang, Harvard, Dr. Eva Maria Novoa Pardo, MIT and Dr. Yingying Huang, MGH/Harvard  
Dr. Nopporn Thangthaeng, USDA, Dr. Murat Keceli, MIT; Dr. Yan X Yu, Brandeis; Chip Allee, CEO CeuticalSoft

4:00 pm

Networking for skills &amp; advice (Sea of learning)

5:00 pm

Closing remark (Dr. Grace Wong, ActoKine Therapeutics, former Genentech)



Dr. Linus Pauling



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Let Nobel-Pauling's candle light up the path to new drug discovery  
www.Nobel-Pauling.org

2013 Nobel-Pauling Biotech Christmas  
Dim Sum Symposium



A moment lasts all of a second, but the memory lives on forever



A smile happens in a flash, but its memory can last a lifetime

## 2012 Nobel-Pauling Biotech Pharma Dim Sum Symposium Agenda

Boston; 2012 Christmas (11 am to 5 pm)

Dear Dr. Grace Wong, Many thanks for hosting an excellent Linus Pauling Biotech Symposium to mix scientists from academia with industry on Christmas holidays. It was a great opportunity for all of us to get to know each other for future collaboration.

Behzad Etemad, PhD, Postdoc, BU School of Medicine, Section of Infectious Diseases (Dec 27, 2012).

Hi Grace: Thank you very much for inviting me to participate the symposium. You have done a wonderful job to organize the Linus Pauling Christmas Biotech Symposium. I am enjoying the meeting very much.

Wenhui Wang, MD, PhD, Professor, Department of Pharmacology, NYMC (Dec 27, 2012)

### Agenda

11:00 am	Registration & Biotech who's who (networking, dim sum from 12 to 3pm)
12:00 pm	Welcome: Dr. Grace Wong, ActoKine Therapeutics Legacy of Dr. Linus Pauling (Richard Shamon, Nobel-Pauling)
12:30 pm	Keynote speakers (Drive the next wave to science innovation) Dr. Qiyong Hu, Novartis; Dr. Lih Ling Lin, Pfizer Prof Wenhui Wang, New York Medical College; Prof Yan Zhu, Tianjin University of TCM (USA vs. China for new drug discovery)  Panel Discussion: Academia vs. Industry for science innovation Moderators: Dr. Jason Deng, MIT & Dr. Junjie Lu, Harvard Dr. Lih Ling Lin, Pfizer; Dr. Qiyong Hu, Novartis; Steven Bodovitz, Aduro BioTech; Dr. Xiang Yang Yu, Ironwoodpharma; Dr. Grace Wong, ActoKine (former Genentech); Prof Wenhui Wang, New York Medical College; Prof Yan Zhu, Tianjin University of TCM; Prof Sami Noujaim, Tufts medical center; Dr. Yazdani B. Shaik Dasthagirisahab, BU
2:00 pm	Smart pitch – Biotech Who's who (the joy of learning)
2:15 pm	Short presentation: Why scientist? The past, the present & the future. Dr. Jason Deng, MIT; Dr. Junjie Lu, Harvard; Dr. Izabela Durzynska, Queens University Belfast, Northern Ireland; Dr. Chuanwu Wang, MIT; Dr. Yanyan Liu, Purdue University; Dr Remi Villenave, Harvard; Dr. Behzad Etemad, BU; Dr. Ravikumar Vasudevan, University of Florida; Dr. Jiayi Zhou, BU; Dr. Weiping Wang, MIT  Smart pitch-Biotech who's who (the joy of sharing) Dr. Bin Ma, BiogenIdec; Dr. Bob Ward, AstraZeneca; Dr. XianLu Qu, Merck; Dr. Shuqi Wang, Harvard Hiromi Miura, SciVax, Japan; Dr. Elichilia Shao, East Africa; Dr. Li Xie, UMass Medical School; Dr. Changyou Zhan, Boston Children's Hospital; Dr. Aoune Barhoumi, MIT; Dr. Steve Arkinstall, EMDSerono; Dr. Mia Wang, Abbott; Julio Vito Wykrota, EincoBio, Brazil; Chip Allee, CeuticalSoft; Katarzyna Kaczmarek, BU; Dominik Conrad, Justus-Liebig University, Giessen, Germany; Anisha korde, Northeastern University; Supriya Demagu, Northeastern University Prakhar Kapoor, University of Massachusetts Lowell; Mansi Soni, University of Massachusetts Lowell
4:00 pm	Networking for skills & advice (Sea of learning)
5:00 pm	Closing remark (Dr. Grace Wong, ActoKine Therapeutics)

The 2011 Linus Pauling Symposium in Boston was a wonderful opportunity and experience for me to see first-hand that the spirit and camaraderie among academic and biopharmaceutical scientists in promoting science is alive and well. It was a terrific way to pay tribute to the late Dr. Linus Pauling. It was a privilege to be part of the event.

Dr. Thomas Tan, Roche NJ (Dec 26, 2011)



## 2011 Nobel-Pauling Biotech Pharma Dim Sum Symposium

Boston, Dec 26, 2011 (11 am to 5 pm)

### Agenda

11:00 am	Registration & who's who (networking, dim sum from 11am to 2pm)
12:00 pm	Welcome: Dr. Grace Wong, CEO, ActoKine Therapeutics, Boston. Legacy of Dr. Linus Pauling: Dr. Zhen Huang, Dept of Chemistry, MIT
12:30 pm	Keynote speakers: Dr. Seng-Lai (Thomas) Tan, Senior Research Leader, Roche, NJ Seminar title: The Biopharma Dilemma: Unprecedented Challenges and Opportunities Dr. Zhijian Lu, Head of Biologics, China Novartis Dr. Li Xing, Senior Principal Scientist, Pfizer Dr Grace Wong, CEO, ActoKine Therapeutics 1st seminar title: AK-1 for Radioprotection & AK-2 for virus prevention 2nd seminar title: Get a foot in the door
2:00 pm	Self Introduction from panel speakers – Who's who
2:30 pm	Panel Discussion: Yin & Yang: lessons learned from Academia and BioPharma Moderators: Dr. Jason Xiang (Executive Director, ChemPartner) & Dr. Paul Yang, MGH (why scientist? The past, the present & the future)  Dr. Thomas Tan, Roche, Senior Research Leader, Hoffman-La Roche, NJ Dr. Zhijian Lu, Head of Biologics, China Novartis Dr. Li Xing, Senior Principal Scientist, Pfizer; Dr. Ju Huang, Postdoc, Harvard Dr. Zhen Huang, Dept of Chemistry, MIT; Dr. Andrej Jedinak, Harvard

Lan Yang, Genzyme; Dr. Bhupendra Shravage, UMass Medical School  
 Dr. Wei Xu, CEO Broadband Photonics; Dr. Hironao Nakayama, Harvard  
 Prof. Libin Cui, Boston U School Med; Dr. Lingge Lu, Harvard Med School  
 Prof. Tara Devi Ashok, UMass Boston; Dr. Ravindra Prajapati, Harvard  
 Dr. Aditya Ambade, UMass Medical School; Dr. Yaguang Si, Agios Pharma  
 Dr. Dapeng Chen & Dr. XianLu Qu, Merck; Dr. Ravindra Prajapati, Harvard  
 Dr. Gene Lee, EMDSerono & Dr. Steve Arkinstall, EMDSerono  
 Dr. Jianguo Yang & Dr. Daotian Fu, Genzyme; Dr. Jiandong Geng, BU  
 Dr. Sridaran Natesan, Scientific Site Head (R&D), Sanofi-Aventis  
 Dr. Mia Wang, Dr. Carolyn Hsu & Dr. David He, Abbott

Dr. Victor Chu, BMS & Dr. Mak Jawadekar, former Pfizer  
 Dr. Gunther Winkler, former BiogenIdec; Nick Leap, Genewiz  
 Dr. B Xia & Dr. D Qin, GSK; Dr. WL Zhou & Dr. J. Cao, Novartis  
 Keven Stevens & Mark Campbell, IDT & Dr. Jorge Andrade, BGI  
 Dr. Xuan Liu, OriGene & Daniel Harris, UMass Boston  
 Dr. Kevin Zhou, CEO, SSTK Biotech, Shenzhen, China  
 Drs. Yajun Liu, CEO, Lisa Shen & George Li, Suzhou  
 Julio Vito Wykrota, Eincobio, Brazil; Dr. Carl Edwards, ex-Amgen  
 Hamilton Lenox, Neuland Lab, India; Dr. Mak Jawadekar, ex-Pfizer  
 Dr. Jeff Wu, MD (BWH); Dr. Steven Levine; Dr. Lex Van der Ploeg, ex-Merck  
 Urs Tanner, Medela, Switzerland; Chip Allee, CeuticalSoft  
 Dr. Zhu Huaxing, CEO, Novoprotein & Patrick Burke, Director, Amarex

4:00 pm	Smart pitch or tell a story: by students/postdocs/scientists
5:00 pm	Closing remark (Dr. Grace Wong, CEO, ActoKine Therapeutics, USA)

Dear Grace, During the 2011 Xmas Pauling symposium, it was a great experience to see how you care for young scientists to flourish and bring out the best in them by linking the students with industry and academic personnel. It was a great honor to be part of this group. I wish you all the best in your endeavors of this kind. Thank you. "Scientific quest is unquenchable, the path most adventurous, what can be more fascinating than knowing every day a new scientific truth. Come, one and all to be initiated into scientific endeavors to be eternally in search of truth." (by Prof. Tara Devi S. Ashok, University of Massachusetts Boston).

Prof Tara Ashok, UMass Boston (Dec 26, 2011).



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### 2011 Xmas Pauling Symposium Speakers



Dr. Linus Pauling



Dr. Paul Yang  
Harvard



Dr. Wei Xu  
Broadband  
Photonics



Dr. Jason Xiang  
ChemPartner



Dr. Grace Wong  
ActoKine





Lan Yang  
Genzyme



Dr. Tara Ashok  
UMASS Boston



Dr. Zhijian Lu  
Novartis



Dr. Li Xing  
Pfizer



Dr. Thomas Tan  
Roche



Dr. Libin Cui  
BU

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**The president of the future - Imagination**

Thanks to Drs Yanjun Liu, CEO, Suzhou, Lisa Shen & George Li and Novoprotein (Dr. Zhu Huaxing, CEO and Dr. Cai Lijun for sponsoring the 2011 Dec 2 Shanghai Nobel-Pauling BioPharma Symposium. Thanks for Prof Yong X. Wang and Prof Dongqing Wei for co-organizing the Linus-Pauling Biotech Pharma Symposium on Dec 2, 2011 at Shanghai Jiao Tong University (SJTU) and Huang Jinlu(黄金路 Gold Road) for volunteering to take photos at the symposium.



Dr. Linus Pauling

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Thanks to Drs Yanjun Liu, CEO, Suzhou, Lisa Shen & George Li  
for sponsoring the 2011 Nobel-Pauling Biotech Symposium



Dr. Lisa Shen  
Suzhou

Prof. Yong X. Wang  
Shanghai Jiao Tong U

Dr. George Li  
Suzhou

Dr. Grace Wong  
ActoKine Therapeutics



Dr. Linus Pauling



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## 2011, Dec 2, Linus Pauling Biotech Pharma Symposium Shanghai Jiao Tong University



Prof Dongqing Wei  
SJTU

Prof Yong X. Wang  
SJTU

Dr. Grace Wong  
ActoKine, USA

Dear Grace, Thank you for taking your precious time to bring so many speakers here to speak at the Shanghai Jiao Tong University (SJTU). Honestly speaking, I learned much more knowledge not only from sciences but also from life. Thanks for teaching us to be useful for the society & the world. Thanks for inspiring us to help our boss succeed and also respect our mentors. Thanks for sharing your exciting science and practical advice. I will need to pick up a lot skills to make myself useful. The advice from the Pauling Symposium speakers has broadened my horizons. For example, how to start, develop and grow a company. I really liked the symposium so much not only because of the free pizza or Chinese food, but because the valuable advice and the opportunity to meet speakers face to face for future jobs or collaborations. I believe others also enjoyed the Pauling symposium very much. It was my pleasure to volunteer to take photos for the symposium. I look forward to taking more photos for the future Nobel-Pauling Biotech Pharma Symposium. Thank you, Gold Road

Jinlu Huang (Gold Road) 黄金路, Student at SJTU, Shanghai (Dec 2, 2011).



**Dr. Linus Pauling**

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**Linus-Pauling Biotech Pharma Symposium**  
**Shanghai Jiao Tong University**  
**Dec 2, 2011**

Gold Road   Qi Xiao   Zhong-wei Chen   Prof Yong X. Wang   Hui Fan   Bin Zhu   Dr. Bin Hao



Yu Jia   Jun Lu   Nian Gong   Yan-chao Wang   Xiao-ling Chen   Dr. Grace   Ai-niu Ma

ActoKine Therapeutics  
Boston, USA

## 2011 Nobel-Pauling Biologics Symposium at Shanghai Jiao Tong University

The Nobel-Pauling Biotech Symposium series is held in honor of the Life and Work of Dr. Linus Pauling, winner of two unshared Nobel Prizes - in 1954 for Chemistry and in 1962 for Peace.

### The goals of the Nobel-Pauling Biotech symposium are:

- To build a biotech bridge between USA, Europe and Asia for new drug discovery
- To encourage biotech collaboration between academia and the biotech industry
- To share practical advice for students, postdocs, scientists and young CEOs

### The topics are:

1. Open Innovation: Academia vs. Industry for new drug discovery.
2. If Innovation is the biotech seed, where is the soil?
3. Ask the experts: how to get a foot in the door.

Date and time: Friday Dec 2, 2011 (2:00 - 10:00 pm)

Fee: \$0 registration at [www.nobel-pauling.org](http://www.nobel-pauling.org)

Location: Shanghai Jiao Tong University, Shuhua Lecture Hall, The Biomedicine Building, 800 Dongchuan Road, Shanghai (Phone: 135-6448-3969).

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### Agenda

2:00pm      Mixing the arts with science: slide presentation through the performing arts 2 seminars by Dr. Grace Wong, CEO, ActoKine Therapeutics, Boston, USA  
 1st seminar: AK-1 for cancer & AK-2 for virus prevention

	2nd Seminar: Biotech: Academia vs. Industry & Get a foot in the door Smart pitch: Lin Huang, Lina Ma, Peisi He, Yukun Wang, Qiang Zhou, Yufang Wang, Huaimeng Fan, Mingzhu Zhao, Shigao Chen, Hao Dai and Xiaolei Cui
4:00 pm	Who's who - Self introduction or smart pitch
5:00 pm	Welcome: Dr. Grace Wong, CEO, ActoKine Therapeutics, Boston, USA
5:10 pm	Introduction: Prof Yong X. Wang & Prof. Dong-Qing Wei, SJTU
5:20 pm	Panel speakers – Biotech Who's who (mixing the biz with science)
6:00 pm	<p>Panel Discussion: Yin &amp; Yang: lessons learned from working many years in BioPharma Moderators: Prof Yong X. Wang and Prof. Dong-Qing Wei, SJTU</p> <p>Dr. Zhenping Zhu, Executive VP, Kadmon Corp. GM, Kadmon, Shanghai            Dr. Wenzhi Tian, CEO, Huabo BioPharma, Shanghai            Dr. Allan Riting Liu, VP, Wanbang Biopharmaceutical Group, China            Dr. Michael Yu, CEO, Innovent Biologics, Shanghai, China            Dr. Cai Lijun, VP and Dr. Zhu Huaxing, CEO, Novoprotein            Dr. Zhijian Lu, Head of Biologics, China Novartis Institutes            Dr. Jason Wong and Dr. Min Wu, Roche, China &amp; Kyung-Hee Jung, Korea</p> <p>Jeff Hou, Australian Institute for Bioengineering &amp; Nanotechnology            Drs. Yanjun Liu, CEO, Lisa Shen and George Li, Biotech, Suzhou            Dr. Jeffrey Su, CSO, Cytovance Biologics, Oklahoma City, USA            Dr. Karen Wen, CEO, Mycenax, Taiwan            Dr. Joerg Lindenblatt, Sartorius, Germany            Dr. Michael Lee, Biomabs Pharma, Shanghai &amp; Akira Hosoki, BioPharma, Japan            Dr. Andrew Racher, Lonza, UK &amp; Sze Guan Chua, Lonza, Singapore</p> <p>Dr. Guoqian Xu &amp; Dr. DQ Wang, Bayer            Dr. Li Feng, CEO, Beijing Mabworks Biotech            Clement Leonard Trono, Tommy Tang, Huang Tao, Genor Biopharma            Dr. Hongfeng Zhou, COO, YZYBio, Wuhan &amp; Dr. Wenyong Wang, SF            Dr. Bo Xu, Wuxi AppTec &amp; Dr. James Ruan, Wuxi PharmaTech            Dr. Howard Hong, Mark Hu, Craig Sng, Parker, Beijing            Dr. Scott Liu, CEO, Henius Biotech &amp; Dr. Kelvin Shao, Newsummit BioPharma            Kevin Zhou, CEO, SSTK Biotech &amp; Dr. Jian Ni, CEO, Human Antibodomics            Prof Jianhua Chang, Fudan, Prof Yuhong Xu, SJTU, Prof. Yong X. Wang, SJTU &amp; Prof. Dong-Qing Wei, SJTU</p>
7:30 pm	Presentation by speakers
9:30 pm	Closing remarks (Dr. Grace, ActoKine Therapeutics, Boston)

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# Molecular Medicine Tri-Conference 2010

Conference: February 3-5 | Exhibits: February 3-4  
Moscone North Convention Center | San Francisco, CA



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## PLENARY KEYNOTES



### When Drug Research is Personal

John F. Crowley, Founder,  
Novazyme Pharmaceuticals, Inc.



### Technology, Aging, and the Brain

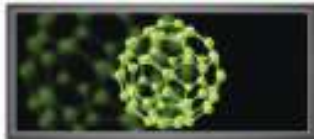
Gary W. Small, M.D., Professor,  
David Geffen School of Medicine,  
University of California,  
Los Angeles



### Chips, Clones and Living Beyond 100

Paul J.H. Schoemaker, Ph.D.,  
M.B.A., Professor,  
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# Molecular Medicine Tri-Conference 2010 CONFERENCE-AT-A-GLANCE

## Tuesday, February 2

8:00 AM	Morning Short Course Registration and Coffee
<b>9:00 - 12:00 PM</b>	<b>Morning Short Courses (Courses 1-6)</b>
10:15 - 10:30	Networking Coffee Break
1:00 - 2:00	Afternoon Short Course Registration
<b>2:00 - 5:00</b>	<b>Afternoon Short Courses (Courses 7-12)</b>
3:15 - 3:30	Networking Refreshment Break
5:00	Close of Day

## Wednesday, February 3

7:00 AM	Registration and Morning Coffee
8:00 - 9:40	Plenary Keynotes
9:40 - 11:00	Grand Opening Refreshment Break in the Exhibit Hall
<b>11:00 - 12:40 PM</b>	<b>Concurrent Channels</b>
12:40 - 1:45	Sponsored Luncheon Presentations or Lunch on Your Own
1:45 - 2:15	Dessert in the Exhibit Hall
<b>2:15 - 4:20</b>	<b>Concurrent Channels</b>
4:20 - 5:20	Reception in the Exhibit Hall ( <i>Sponsorship Available</i> )
5:20 - 6:20	Break-out Discussions
6:20	Close of Day

## Thursday, February 4

<b>8:25 - 10:30 AM</b>	<b>Concurrent Channels</b>
10:30 - 11:30	Poster Competition, Refreshment Break & Raffles in the Exhibit Hall
<b>11:30 - 12:30 PM</b>	<b>Concurrent Channels</b>
12:30 - 1:45	Sponsored Luncheon Presentations or Lunch on Your Own
1:45 - 2:15	Ice Cream Refreshment Break in the Exhibit Hall
2:15 - 3:05	Plenary Keynote Session
3:05 - 3:45	Refreshment Break in the Exhibit Hall
<b>3:45 - 5:50</b>	<b>Concurrent Channels</b>
5:50	Close of Day

## Friday, February 5

<b>8:30 - 10:20 AM</b>	<b>Concurrent Channels</b>
10:20 - 11:00	Coffee Break
<b>11:00 - 12:00 PM</b>	<b>Concurrent Channels</b>
12:00 - 1:00	Sponsored Luncheon Presentations or Lunch on Your Own
<b>1:00 - 3:05</b>	<b>Concurrent Channels</b>
3:05	Close of Conference

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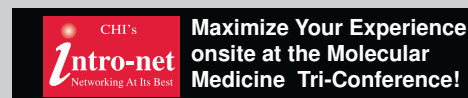


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# PRE-CONFERENCE SHORT COURSES\*

Tuesday, February 2

\*Separate Registration Required

## MORNING COURSES: 9AM – 12PM

### (SC1) APPLYING NEXT GENERATION SEQUENCING TECHNOLOGIES TO RESEARCH

*Introduction to New Technologies and Application in Research*

- Technologies for newest platforms for next generation sequencing
- Strategies and tools for managing data
- Demonstration of how tools can be applied to research

*Course Moderator:*

Stanley Gloss, *Founding Partner Managing Director, BioTeam, Inc.*

*Course Instructors:*

Francisco M. De La Vega, D.Sc., *Distinguished Scientific Fellow, Computational Genomics Research, Genetics Systems R&D, Life Technologies*

Giles Day, *Senior Director, BBC Informatics, Pfizer Biotherapeutics & Bioinnovation Center*

Ronald W. Davis, Ph.D., *Professor, Biochemistry & Genetics, and Director, Stanford Genome Technology Center, Stanford University*

### (SC2) ONE CASE STUDY IN BREAST CANCER- THREE PERSPECTIVES

*Illustrating Current Challenges in Personalized Medicine*

- Overview of case study
- Clinical perspective
- Diagnostic and biomarker perspective
- Payer perspective

*Course Instructors:*

Michael Liebman, Ph.D., *Managing Director, Strategic Medicine, Inc.*

Laura J. Esserman, *Professor, Surgery, University of California, San Francisco Medical Center*

Tracey Colpitts, Ph.D., *Manager, Abbott Molecular*

### (SC3) MIGHTY MITOCHONDRIA: Their Relevance to Disease and Translational Medicine

- Mitochondria in disease and drug induced toxicity (James Dykens)
- Assessing mitochondrial function preclinically (Yvonne Will)
- Non-invasive mitochondrial assessment in the clinic (Robert Wiseman)

*Course Leader:*

Yvonne Will, Ph.D., *Compound Safety Prediction- WWMC, Cell Based Assays and Mitochondrial Biology, Pfizer R&D*

*Course Instructors:*

James Dykens, Ph.D., *Drug Safety R&D, Pfizer Inc*

Yvonne Will, Ph.D., *Compound Safety Prediction- WWMC, Cell Based Assays and Mitochondrial Biology, Pfizer R&D*

Robert Wiseman, Ph.D., *Associate Professor, Department of Physiology, Michigan State University*

### (SC4) ADDRESSING SAFETY CONCERNS FOR BIOLOGICAL DRUGS

- Overview of challenges pertaining to safety for biologics
- Safety assessments at pre-clinical and clinical stage
- Use of new assays, animal models and biomarkers for early predictions
- Regulatory guidelines and their interpretations

*Course Instructors:*

Hong Wang, Ph.D., *DABT, Safety Assessment, Genentech Inc.*

Kathleen Meyer, MPH, Ph.D., *DABT, Senior Director, Preclinical Safety Evaluation, XOMA (US) LLC*

### (SC5) TARGETING CANCER STEM CELLS WITH BIOLOGICS

- Novel nanoparticle fusion proteins, tr1 and tr4, that achieve normal p21 delivery to p53/p21 mutated tumors (tr1) and inhibition of notch signaling (tr4) resulting in tumor eradication
- Differentiation versus self-renewal: changing cancer stem cell fate by targeting stem cell pathways
- Cancer stem-like cells: isolation using biological criteria and use in drug discovery and development
- Cd47: an adverse prognostic factor and therapeutic antibody target on human acute myeloid leukemia stem cells
- Identification of stem cell markers in the normal prostate and prostate cancer

*Course Instructors:*

Agamemnon Epenetos, Ph.D., *FRCP, Chairman, Trojantec Ltd.*

Austin Gurney, Ph.D., *Vice President, Molecular and Cellular Biology, OncoMed Pharmaceuticals, Inc.*

Jennie P. Mather, Ph.D., *Senior Vice President, Stem Cell Research, MacroGenics, Inc.*

Ravi Majeti M.D., Ph.D., *Assistant Professor, Division of Hematology, Stanford Cancer Center, Institute for Stem Cell Biology and Regenerative Medicine*

Kevin G. Leong, Ph.D., *Scientist, Tumor Biology and Angiogenesis, Genentech, Inc.*

### (SC6) BLOOD-BRAIN BARRIER

- The physiological basis for the "barrier" nature of the BBB
- Experimental approaches (*in vitro/in vivo*) that are available for screening for brain penetration
- Medicinal Chemistry perspective on *in vitro/in silico* approaches for optimizing CNS penetration
- Multi-parameter optimization (MPO) for CNS penetration
- *In vivo* examples where all these concepts are applied together, e.g., consideration of free fractions in various compartments in relation to *in vitro* pharmacology values
- Projecting human receptor occupancies considering species differences in affinity, free fraction
- Exposure targeting for biomarker studies

*Course Instructors:*

Christopher L. Shaffer, Ph.D., *Associate Research Fellow, Pharmacokinetics, Dynamics & Metabolism, Pfizer, Inc.*

Douglas Spracklin, Ph.D., *Director, Pharmacokinetics, Dynamics and Metabolism, Pfizer, Inc.*

Travis T. Wager, Ph.D. *Associate Research Fellow, Neuroscience Discovery, Medicinal Chemistry, Pfizer, Inc.*

## AFTERNOON COURSES: 2PM – 5PM

### (SC7) BEST PRACTICES IN TRANSLATIONAL & PERSONALIZED MEDICINE

- Real world solutions currently in place in pharma, national labs, academia, and industry
- Building collaborations and sharing biological data between Big Pharma
- Bridging the gap between bench and bedside
- Informatics solutions that link data from the clinic with cutting edge research

*Course Instructors:*

Jeffrey S. Barrett, Ph.D., *FCP, Pediatrics Director, Pediatric Pharmacology Research Unit, The Children's*

*Hospital of Philadelphia*

Lisa LaLuna, *Senior Vice President, Corporate Development & Implementation, ePharmaSolutions*

Jeremy Packer, *Head, Bioinformatics, Abbott*

Faye D. Schilkey, *Associate Director, NM Sequencing Center, National Center for Genome Resources*

### (SC8) STRATEGIES FOR MOLECULAR DIAGNOSTIC COMPANIES

*Achieving Success in Rapidly Changing Markets*

- Why and how diagnostics markets have changed
- Strategies for success: conventional or new markets?
- Major business model questions including partnering
- How to obtain your next round of funding

*Course Instructors:*

Keith F. Batchelder, *Chief Executive Officer, Genomic Healthcare Strategies*

Peter S. Miller, *Chief Operating Officer, Genomic Healthcare Strategies*

### (SC9) FRAGMENT-INSPIRED MEDICINAL CHEMISTRY

- Fragment-based approaches as platforms for medicinal chemistry
- Fragment-based methods that inspire fresh approaches to lead generation
- Optimization of fragment hits
- Combining technology with fragment-based methods to advance medicinal chemistry
- Facing the challenge of applying fragment-based approaches when structural information is not available
- Promises and pitfalls of surface plasmon resonance (SPR) for fragment methods

*Course Instructors:*

Michelle Arkin, Ph.D., *Associate Director, Biology, Small Molecule Discovery Center, Pharmaceutical Chemistry, University of California, San Francisco*

Daniel A. Erlanson, Ph.D., *Co-founder, Carmot Therapeutics, Inc.*

### (SC10) TRANSPORTER-MEDIATED DRUG-DRUG INTERACTION POTENTIAL

*Strategies for in vitro Characterization*

- Clinical relevance of transporter DDI's
- *In vitro*, cell based models for evaluating transporter interactions of substrates and inhibitors
- Case study: minimizing p-glycoprotein interactions as a barrier to CNS penetration

*Course Instructors:*

Phil Burton, Ph.D., *Chief Executive Officer & Chief Scientific Officer, ADMETRx, Inc.*

Xingrong Liu, Ph.D., *Senior Scientist, DMPK, Genentech, Inc.*

Joseph A. Ware, Ph.D., *Senior Scientist, Clinical Pharmacokinetics and Pharmacodynamics, Development Sciences, Genentech, Inc.*

### (SC11) BASIC IMMERSION: CUTTING EDGE SCIENCE & TECHNOLOGY FOR BIOTECH & PHARMA

- Gain a fundamental understanding of the science and technology driving the Biotech/Pharma industry
- Learn basic scientific terminology used by researchers in the life sciences
- Designed for the non-scientist working with or in the biotech/pharma industry
- Immersion course on the biotech basics; Recombinant DNA, Proteins, Stem Cells, Biologics, Drug Discovery and Drug Development

*Class Materials Include: The Primer: A Biotechnology Guide for Non-Scientists*

*Course Instructor:*

Karin Lucas, Ph.D., *BioTech Primer Instructor and Scientific Advisor*

### (SC12) DESIGNING RIGOROUS OMICS STUDIES FOR BIOMARKER DISCOVERY AND DEVELOPMENT OF PROGNOSTIC AND PREDICTIVE MOLECULAR DIAGNOSTICS

- Why study design is decisive for success or failure
- Critical review of examples
- Dos and don'ts
- A roadmap to the answers
- Samples: How many are enough?
- Apples and Oranges: Tackling confounding factors
- Companion diagnostics in clinical trials
- The regulatory perspective

*Course Instructors:*

Terry Speed, Ph.D., *Professor, Department of Statistics, University of California, Berkeley*

Juergen von Frese, Ph.D., *Managing Director, Data Analysis Solutions, DA-Sol GmbH*

Donna Roscoe, Ph.D., *Senior Reviewer, FDA/OIVD/DIHD*

# Cambridge Healthtech Institute's Seventh Annual Molecular Diagnostics: Next Wave of Personalized Medicine

Industry leader's  
networking event

**WEDNESDAY, FEBRUARY 3**

**7:00 AM Registration and Morning Coffee**

**8:00 Plenary Keynote Session** (See Page 26 for Details)

**9:40 Grand Opening Refreshment Break in the Exhibit Hall**

## KEYNOTE PRESENTATIONS

### 11:00 Chairperson's Remarks

*Harry Glorikian, Managing Partner, Scientia Advisors*

### 11:05 Molecular Diagnostics as a Value Driver of Pharma/ Pharma as a Value Driver for Molecular Diagnostics

*Michael C. Little, Global Head, Diagnostics Development, Novartis Molecular Diagnostics*  
Molecular Diagnostics is a critical success factor for the future of pharmaceuticals and an essential aspect of the move toward personalized medicine. At the same time, to progress better healthcare and patient outcomes, it is imperative that the pharma industry's understanding of both targeted drug discovery and drug commercialization is fully leveraged to enable innovative diagnostics to be put into clinical practice and influence physician decision-making. The keynote will focus on these elements and discuss 1-2 case studies of how we at Novartis are using our discovery and development approach to work toward bringing innovative companion and stand-alone diagnostic tests to market.

### 11:40 Building a Successful Diagnostics Business Model in the Era of Personalized Medicine

*Richard Ding, CEO, bioTheranostics, a bioMerieux Company* Personalized medicine has been generally accepted as an inevitable trend in healthcare. However, much debate is still ongoing related to a sustainable business model for diagnostics companies in this new space. This presentation will identify various challenges, risks and potential returns for diagnostic companies, explore partnership models and propose some basic framework to seize the growth opportunity of personalized medicine.

### 12:15 PM Personalized Medicine: It Takes a Village

*Mark Stevenson, President & COO, Life Technologies Corp.*

New technologies, such as next generation sequencing, can be rapidly adopted in the research labs and help breakthroughs in our understanding of disease mechanisms for personalized medicine. But the journey from research technology to diagnostic systems is challenging and slow. As our understanding of disease increases the promise of personalized medicine is coming closer but what will it take to cross the bridge from research tool to routine diagnostics in personalized medicine. The Presentation will focus on the journey Life Technologies has embarked on and the partnerships and collaborations necessary to translate the tools for the research lab into solutions personalized medicine.

### 12:50 Luncheon Presentation

#### Information Trends in Biomarker Research

*Colin Williams, Ph.D., Director, Product Strategy, Thomson Reuters Healthcare and Science*

In recent years, the quantity of data published on biomarker research has exploded. The challenge faced by researchers is to find vital, relevant information on the best biomarker quickly and reliably. In this discussion we will introduce BIOMARKERcenter, a comprehensive, fully-indexed biomarker information resource, and through case studies show how it aids the discovery process.

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### 1:45 Dessert in the Exhibit Hall

## MOLECULAR DETECTION OF PATHOGENS: MEETING THE NEEDS OF THE COMMUNITY

### 2:15 Chairperson's Remarks

#### 2:20 Real-time Detection and Characterization of Influenza: The Need for Speed

*Karen L. Kaul, M.D., Ph.D., Board of Directors Chair of Molecular Pathology, Director, Molecular Diagnostics Division, Director, Pathology Residency Program, NorthShore University HealthSystem; Clinical Professor of Pathology, University of Chicago Pritzker School of Medicine*

The Novel H1N1 Influenza outbreak of 2009 challenged laboratories and hospitals with the need for high volume testing and rapid resulting in order to appropriately treat and isolate infected patients. Molecular approaches offer clear advantages, though no pre-validated assays were available for this unanticipated virus. This presentation will address various assays for detection and differentiation, as well as other laboratory issues, and will review the recent outbreak from the laboratory perspective.

#### 2:50 What Happened with SARS: Lessons Learned and Applied to Influenza

*Joseph D. Miller, Ph.D., Chief, Laboratory Preparedness Officer, Influenza Division, Centers for Disease Control and Prevention*

#### 3:20 MRSA Surveillance Programs – What Impacts Success?

*Lance R. Peterson, M.D., FASCP, FIDSA, Director, Microbiology & Infectious Disease, Evanston Hospital, NorthShore University HealthSystem and University of Chicago*  
Control of any epidemic relies on detection of those harboring the pathogen (infected and colonized). For any MRSA prevalence, the operational processes most influential are 1) sensitivity of the laboratory methods used, 2) speed at which unknown positive patients are detected, and 3) the selection of who is to undergo screening. The current understanding of these specifics will be presented.

#### 3:50 Real-time Array PCR for Infectious Diseases(RAP-ID): Merging Multiplex PCR and Real-Time Microarray Detection in a Single Tube for Sensitive Parallel Genotyping of Pathogens and Antibiotic Resistances

*Wilhelm Pluester, Ph.D., CEO, Eppendorf Array Technologies S.A.*

RAP is a novel hybrid technology combining major advantages of microarrays (multiplexing, specificity) and real-time PCR (sensitivity, dynamic range). Target amplification and hybridization of amplicons proceed in a single tube (in the same buffer) resulting in a simplified, automated workflow with minimal hands-on time. First results in multiplex detection of pathogens and antibiotic resistances associated with ventilator-associated pneumonia are presented.

Sponsored by  
**eppendorf**  
— Array Technologies —

#### 4:05 Platform Genometrica: Novel Instrumentation

#### for Molecular Biology and Medicine

*Vera Gorfinkel, Ph.D., Associate Professor, SUNY SB, Research consultant, Genometrica Corporation*

The technology platform Genometrica aims to develop methods and instruments capable of carrying out on equal footing inexpensive and highly accurate genomic studies including DNA sequencing, hybridization, and quantitative PCR assays. The session will focus on the basic principles, novel engineering solutions, data acquisition/handling methods, and unique research capabilities offered by the Genometrica platform.

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**genometrica**

4:20 Reception in the Exhibit Hall (Sponsorship Available)

## 5:20 BREAK-OUT DISCUSSIONS in the Exhibit Hall

### Consumer Diagnostics (Not Just Genomics) – Get Used to It!

Co-Moderators: Peter S. Miller, Chief Operating Officer, and Keith F. Batchelder, Chief Executive Officer, Genomic Healthcare Strategies

- Technology improvements and cost reductions will make genetic, metabolic, and proteomic analysis cheaper
- Consumers will remain interested and companies will develop better ways of reaching the public
- Providers of traditional care will have to come to grips with informed consumers
- How will this happen?

### Is Economics Going to Be the Driver of Molecular Diagnostics Adoption?

Moderator: David S. Lester, Ph.D., Vice President, Human Health Solutions, Theranos

- Are molecular diagnostics going to make healthcare cheaper and/or better?
- What are the barriers for adoption of molecular diagnostics?
- Payers: US vs. international challenges.
- How will value of the diagnostic be determined?

### Patents and Diagnostics Development: Help or Hindrance?

Moderator: Frances Toneguzzo, Ph.D., Director, Office of Corporate Sponsored Research and Licensing, Massachusetts General Hospital

- Type of patents in the diagnostic space and IP fragmentation
- Other types of intellectual property protection in diagnostics development
- Differentiation in diagnostics and use of patents
- Strategies for effective use of intellectual property to stimulate diagnostic development

### Making Molecular Diagnostics Work Now? The Trials and Tribulations of Labs and Money

Moderator: Ian S. Millett, Ph.D., RAC, Senior Consultant, Medical Devices, Biologics Consulting Group, Inc.

- How do you get paid for a molecular diagnostic test?
- Does implementing a molecular diagnostic test in your lab really make sense?
- Life at Ground Zero - The FDA's changing perspective on lab-developed tests
- Pushing and Pulling- Who is your customer and just how Personal can you make that bill?

### Strategies for Commercialization of Molecular Diagnostics

Moderator: Harry Glorikian, Managing Partner, Scientia Advisors

6:20 Close of Day

## THURSDAY, FEBRUARY 4

### HEALTH IT: WHY IS IT SO HARD?

#### 8:25 AM Chairperson's Remarks

Wayne A. Rosenkrans, Jr., Ph.D., Distinguished Fellow, MIT Center for Biomedical Innovation; Program in Ethics and Systems Medicine, Georgetown University; Chairman, Personalized Medicine Coalition; VP, Strategic Consulting, Fuld & Co.; Chief Scientific Advisor, Expertech Solutions; and Chief Applications Officer, SciTech Strategies

#### 8:30 Keynote Presentation

##### Ensuring Responsible Testing through Real-Time Collaboration Between Providers, Payors and Labs

Matthew B. Zubiller, VP and General Manager, Advanced Diagnostics Management, McKesson Corp.

As molecular diagnostics proliferate, ensuring responsible testing becomes more complex. This is further complicated by changing reimbursement policies and health care IT reform. Ensuring your lab's success requires technology-enabled collaboration with providers, payors and other labs. This keynote discusses business practices and strategies for labs to offer decision support and access to a broader array of tests to providers, to review payors' reimbursement policies before tests are performed and to build effective lab networks to fulfill orders.

#### 9:15 Keynote Presentation

##### HIT and PM: Conflict or Convergence

Wayne A. Rosenkrans, Jr., Ph.D., Distinguished Fellow, MIT Center for Biomedical Innovation; Program in Ethics and Systems Medicine, Georgetown University; Chairman, Personalized Medicine Coalition; VP, Strategic Consulting, Fuld & Co.; Chief Scientific Advisor, Expertech Solutions; and Chief Applications Officer, SciTech Strategies

### 10:00 The Development of Multigene Prognostic and Predictive Tests in Cancer

Austin Tanney, Ph.D., Scientific Liaison Manager, Almac Diagnostics

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The key to the delivery of personalized medicine is the development of molecular diagnostics to improve patient care, from better diagnostic and prognostic tests to companion diagnostics. The use of multigene signatures is increasingly of interest however there are many considerations in developing such signatures from study design to development of deliverable tests. Here we present our experience and perspective.

#### 10:15 Sponsored Presentation (Sponsorship Opportunity Available)

#### 10:30 Poster Competition Refreshment Break & Raffles in the Exhibit Hall

#### 11:30 Expert Panel

##### Health IT: What Will Success Look Like?

Moderator: David S. Lester, Ph.D., VP, Human Health Solutions, Theranos

- Reducing healthcare costs
- Facilitating the adoption of molecular diagnostics
- Defining the goals
- Realizing solutions for reaching the goals

Panelists:

Brandon Savage, M.D., Chief Medical Officer, GE Healthcare

Vance Vanier, M.D., Chief Medical Officer, Navigenics, Inc.

Mark N. Blatt, M.D., MBA, Director, Healthcare Industry Solutions, Digital Health Group, Intel Corporation

Jeffrey D. Miller

#### 12:30 PM Luncheon Presentation (Sponsorship Opportunity Available) or Lunch on Your Own

#### 1:45 Ice Cream Refreshment Break in the Exhibit Hall

### PLENARY KEYNOTE SESSION

#### 2:15 Plenary Keynote Introduction

#### 2:25 Plenary Keynote Presentation (See Page 26 for Details)

#### 3:05 Refreshment Break in the Exhibit Hall

### IP ISSUES ON GENE PATENTING: WHAT IS THE SOLUTION?

#### 3:45 Chairperson's Remarks

Jorge A. León, Ph.D., President, Leomics Consulting

#### 3:50 Gene Patents, Perspectives from the Clinical Laboratory

Karen P. Mann, M.D., Ph.D., Associate Professor and Director, Molecular Hematopathology, Department of Pathology and Laboratory Medicine, Emory University Hospital; President Elect, Association of Molecular Pathology

Gene patents are controversial, but are a reality in molecular diagnostics. As a laboratorian who co-directs an active clinical molecular diagnostics laboratory, I will describe how gene patents affect how I practice medicine including the effect of patents on test menus, turnaround times, choice of laboratories for referral testing, and laboratory finance.

#### 4:20 Gene Patents in Molecular Diagnostics: Valuable Assets or Impediments?

Frances Toneguzzo, Ph.D., Director, Office of Corporate Sponsored Research and Licensing, Massachusetts General Hospital

Increasingly, genetic diagnostics are making use of panels of genes/gene fragments for accurate diagnosis of drug responses (toxicity and/or effectiveness and/or dosing) and disease stratification. In a number of situations, the patents covering these genes or their use are held by different owners, including companies, academic institutions and private individuals/foundations. While patents are generally considered valuable in that they provide a period of exclusivity when a company can exclude others from practicing the patented invention and thus protect the investment the company is making in commercializing the invention, this fragmentation of the intellectual property landscape in molecular diagnostics may impede the development of certain tests.



#### 4:50 IP Fragmentation in Genetic Diagnostics

Jorge Goldstein, Director, Biotechnology & Chemical Group, Sterne Kessler Goldstein Fox PLLC

So called "patent thickets" have appeared when multiple patent owners each control one or a few genetic diagnostic correlation patents for one disease, where the multiple patents cover either alterations in one gene or several genes involved in the disease. Such thickets have already resulted in a failure to offer commercial tests for all possible gene alterations, or have generated test designs that are driven primarily by IP concerns. This talk will discuss possible solutions to the problem, including the use of patent pools driven by medical standards.

#### 5:20 Expert Panel: Bioscience Patent Law

- How do IP fragmentation and patent pools affect the clinical labs and end users?
- How do they affect companies that own IP and commercialize IVDs?
- How do they affect companies and academic that discover new markers?
- Why are some licensing models slowing down the advancement of molecular diagnostics?

#### 5:50 Close of Day

### FRIDAY, FEBRUARY 5

## U.S. UNIVERSAL HEALTH - BREAKING NEWS

#### 8:30 AM Chairperson's Opening Remarks

Brian T. Edmonds, Ph.D., Research Advisor, Global External Research & Development, Lilly Corporate Center

#### 8:35 Regulatory Considerations for Companion Diagnostics and Personalized Medicine

Elizabeth Mansfield, Ph.D., Senior Genomics Advisor, Office of the Chief Scientist; Director, Personalized Medicine, Office of in Vitro Diagnostic Device Evaluation and Safety, Food & Drug Administration

Advances in genomics-based discovery and therapeutic agent targeting have led to greatly increased interest in development of diagnostic/therapeutic combinations that promise to deliver "personalized" therapy to patients. With this vision comes the realization of the importance of the diagnostic test performance upon which the therapeutic safety and efficacy will rest. This presentation of regulatory issues for companion diagnostic devices and codevelopment will address proposed regulatory pathways for the diagnostic device, and emphasize the need for adequate analytical and clinical validation.

#### 9:05 Value Based Laboratory Tests -- What Went Wrong on the Way to the Fair?

Ian S. Millett, Ph.D., RAC, Senior Consultant, Medical Devices, Biologics Consulting Group, Inc.

In spite of tremendous interest, expenditures, and pro-active work by industry, academia, and government, the personalized health care revolution seems to have stalled in the area of new test development and use. This talk will focus on potential reasons for slow uptake of new diagnostic technology, will survey the upcoming landscape, and will remind participants that in the end, it was the tortoise that won the race.

#### 9:35 Medco Personalized Medicine: Advancing Healthcare

Lon Castle, M.D., Senior Director, Personalized Medicine, Medco Health Solutions, Inc.

Pharmacogenomic tests bring a new level of precision to pharmaceutical care, enabling treatment that is targeted to the unique genetic characteristics of individual patients. These tests are becoming the standard of care in many therapeutic areas, as physicians and payers become more conversant with the value of testing. Medco's Personalized Medicine healthcare can improve outcomes and reduce the overall costs of care.

#### 10:05 Innovations in Molecular Diagnostics and SamplePreparation Methods: Accelerating Sample-to-Result Diagnostics

Kevin Banks, Ph.D., Head of Marketing and Sales, Akonni Biosystems

Akonni Biosystems (Frederick, MD) was founded in 2003 and has over 20 patents with 13 others pending. The company's core platform utilizes a gel-drop array technology optimized for developing medical applications, with an emphasis on greatly accelerating the time from sample to result. This session will provide an introduction and overview of the technology and platform. Preliminary results with a number of clinical applications will be discussed.

#### 10:20 Coffee Break

#### 11:00 Economics of Having Diagnostics Reimbursed and the Practical Challenges in Getting Reimbursement

Philip C.M. Ma, Ph.D., Director, McKinsey & Company, Inc.

The impact of diagnostics in influencing care continues to grow with technology advancements in clinical genomics and other molecular markers. In spite of this, the current reimbursement system in the U.S. does not appropriate incentives for effective use - both over and under-use of diagnostics can result. This talk will review how mis-aligned incentives can result from the under-lying micro-economics of different stakeholders (physicians, patients, payors, and diagnostic manufacturers), and will suggest a few ways to improve the micro-economic situation.

#### 11:30 Panel Discussion: Personalized Medicine and Challenges for Implementation

- How will universal healthcare impact your business?
- How will it impact molecular diagnostics adoption?

#### 12:00 PM Luncheon Presentation (Sponsorship Opportunity Available) or Lunch on Your Own

#### 1:00 Chairperson's Remarks

### VALUE OF CONSUMER BASED GENOMICS: WHAT IS THE CONSUMER GOING TO DO WITH IT?

- How good is the information delivered by these tests?
- How well can people understand the results?
- How effectively can they help people manage their health?

#### 1:05 Consumers and Their Genomes

Brian Naughton, Ph.D., Founding Scientist, 23andMe

Over 30,000 individuals now have access to their personal genetic information through 23andMe's web-based services. Consumers sign up for these services to learn about their disease risk or carrier status, to discover their ancestral roots, to find new relatives, or to participate in research on a particular disease such as Parkinson's. This presentation will discuss the ongoing studies that are beginning to reveal how people respond to their personal genetic information.

#### 1:35 Talk Title to be Announced

Patrick F. Terry, CEO, Technic Solutions, LLC; Acting CEO, Grand Therapeutics, Inc.

#### 2:05 Drinking from the Fire Hose: Are Consumers Ready?

Sharon Terry, MA, President and CEO, Genetic Alliance

#### 2:35 Panel Discussion and Q&A with Audience

#### 3:05 Close of Conference

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**Akonni**  
Biosystems

3:50 Presentation Sponsored by 

### Isotopic Mass Tags for the Facilitated Development of Multiplex SRM Mass Spectrometric Assays for Protein and Peptide Biomarkers

Helen Byers, Ph.D., Principal Research Scientist, Proteome Sciences plc, UK

Fit-for-purpose assays are essential for biomarker qualification. Selected Reaction Monitoring (SRM) is increasingly used for the quantitation of peptides and proteins, but is limited by expense and delay connected with synthesis of isotope-doped standard peptides and the difficulty to synthesize more complex standards (e.g. with post-translational modifications). Proteome Sciences has developed isotopic versions of its proprietary tandem mass tag (TMT) reagents to differently label sample and standard allowing to establish TMT-SRM, a method that allows the use of synthetic or natural reference standards to establish assays for any peptide or protein in any given sample material..

- Criteria

4:20 Reception in the Exhibit Hall (Sponsorship Available)

5:20 BREAK-OUT DISCUSSIONS in the Exhibit Hall

### Personalized Medicine: Commercial Hurdles to Adoption in an Era of Evidence Based Medicine

Moderator: Katherine Tynan Ph.D., Business Development & Strategic Consulting for Diagnostics Companies, Tynan Consulting LLC

- The evidence required for each of the stakeholders: analytical performance and clinical validity for the FDA, clinical utility for physicians, and medical necessity for payers.
- The strategic importance of "intended use statements" in guiding product/test development
- The opportunities and challenges with reimbursement

### Development and Application of Assays in the 3D Format

Moderator: Ray Mattingly, Ph.D., Associate Professor, Pharmacology, Wayne State University

- Challenges in standardization and feasibility of 3D cultures
- Development of 3D format for high-throughput assays
- Protocols for drug screening in 3D culture format
- Advanced 3D co-culture approaches to model tissues

### How Innovative Technologies Are Selected, Evaluated, and Translated for Application in Diagnostics for Personalized Medicine to Enhance or Replace Conventional Diagnostics

Moderator: Kewal K. Jain, M.D., Professor, CEO, Jain PharmaBiotech

- The ideal molecular diagnostics laboratory for personalized medicine
- Selection, evaluation, and translation of new diagnostic technologies for personalized medicine
- Role of sequencing
- Future prospects of diagnostics for personalized medicine: supplementing, enhancing or replacing conventional diagnostics

### Biomarkers of Efficacy

Moderator: Tracey Colpitts, Ph.D., Business Development, Companion Products, Abbott Molecular

- How does mechanism of action hypotheses translate to population science?
- When and how do we gather prevalence data?
- What priorities should we be making?

### Next Generation Sequencing in the Clinical Diagnostics Laboratory

Moderator: Karl V. Voelkerding, M.D., Associate Professor of Pathology, University of Utah and Medical Director for Advanced Technology, ARUP Laboratories

- What are the first diagnostic applications that next generation sequencing will be used for?
- What improvements would facilitate translation of the technology into the clinical laboratory?
- How will laboratories process and interpret the large amounts of data generated?

6:20 Close of Day

## THURSDAY, FEBRUARY 4

### PERSONALIZING THERAPY: SERUM BIOMARKERS

#### 8:25 AM Chairperson's Remarks

Josip Blonder, M.D., Sr Research Scientist, Head, Quantitative Proteomics, NCI, Frederick

#### 8:30 Personalized Oncoproteomics for Cancer Biomarker

##### Discovery: Application to Renal Cell Carcinoma

Josip Blonder, M.D., Sr. Research Scientist, Head, Quantitative Proteomics, NCI

Discovery of diagnostic, therapeutic and prognostic markers is central to personalized treatment of cancer. Thus, proteomic approaches capable of characterizing the patient's tumor phenotype using clinically relevant specimens are critically needed. A method that relies on tissue-directed oncoproteomics is described and applied for cancer biomarker discovery in the plasma of a patient diagnosed with renal cell carcinoma.

#### 9:00 Protein Quantification Through Targeted Mass Spectrometry: The Way Out of Biomarker Purgatory?

Steven A. Carr, Ph.D., Director, Proteomics, The Broad Institute of MIT and Harvard

Immunoassays are widely used to measure protein biomarkers in patient blood, but useful antibody reagents do not exist for the vast majority of proteins. We are addressing this serious barrier by developing targeted assay methods employing mass spectrometry to screen and quantify low abundance proteins in plasma. This presentation will focus on the latest developments and applications of these technologies.

#### 9:30 Mass Spec for Prostate Biomarkers, Assessing Aggressive vs. Non-Aggressive Prostate Cancer

Jianfeng Xu, M.D., Ph.D., Professor, Epidemiology, Prevention and Cancer Biology, Director, Ctrr for Cancer Genomics, Wake Forest University School of Medicine

Three types of prostate cancer related genetic variants have been found from genome-wide association studies, including those associated with overall prostate cancer risk, aggressive prostate cancer risk, and higher baseline PSA levels. These genetic variants may have potential clinical utility. However, further studies are needed to assess their clinical validity and clinical utility.

#### 10:00 A Novel Tool for Non-Invasive Disease Detection

Jack Leonard, Ph.D., Vice President of Technology Commercialization, febit Inc.

Sponsored by



We developed a novel non-invasive diagnostic assay based on microRNAs. Our Biomarker Signature assay has shown an outstanding performance for the integrative detection of a broad panel of diseases and is well suited for high sample throughput at low cost since for each test less than one minute hands-on time is required. Moreover, our approach stands-out by high reproducibility and sensitivity while test-to-test variations are minimal.

#### 10:15 Metabolite Profiling: Opportunities for Identification and Validation of Novel Biomarkers

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metanomicshealth   
Hajo Schiewe, Ph.D., Senior Manager, Business Development, Metanomics Health

Metabolite profiling is the parallel measurement of a broad range of endogenous and xenobiotic metabolites in a given biological sample. The metabolome reflects internal or external influences on the pathophysiology of an organism including drug treatment and disease status. Metanomics Health uses mass spectroscopy based metabolite profiling to identify and validate novel metabolite biomarkers for a range of applications in pre-clinical and clinical drug development, disease diagnostics and progression. The analysis and interpretation of metabolite changes can increase the mechanistic understanding of diseases, drugs and other influences on an organism.

# Cambridge Healthtech Institute's Inaugural Personalized Diagnostics: "Under the Hood" Technologies for Molecular Diagnostics

WEDNESDAY, FEBRUARY 3

7:00 AM Registration and Morning Coffee

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Information Trends in Biomarker Research

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Thomson Reuters Healthcare and Science

In recent years, the quantity of data published on biomarker research has exploded. The challenge faced by researchers is to find vital, relevant information on the best biomarker quickly and reliably. In this discussion we will introduce BIOMARKERcenter, a comprehensive, fully-indexed biomarker information resource, and through case studies show how it aids the discovery process.

Sponsored by



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## PERSONALIZING THERAPY: TISSUE BIOMARKERS

2:15 Chairperson's Remarks

Linda McAllister, M.D., Ph.D.

2:20 Relating Biomarkers to Efficacy: The Efficacy Curve

Tracey Colpitts, Ph.D., Manager, Abbott Molecular

A method of predicting response in a subgroup defined by a biomarker will be discussed and demonstrated using data from therapeutic trials involving EGFR inhibitors in lung, colon, and breast cancer. Biomarkers that aid in selecting subgroups of patients of response were analyzed and compared. Striking similarities between the different cancers, therapies, and subgroups reveals a relationship between biomarkers and efficacy, which is visualized in the efficacy curve.

2:50 Network and Pathway Analysis of a Novel 3D Breast Carcinoma Model by Both Digital Gene Expression (DGE) and Whole Genome Array Analyses

Ray Mattingly, Ph.D., Associate Professor, Pharmacology, Wayne State University

We have developed a tractable, *in vitro* model of ductal carcinoma *in situ* (DCIS) based on 3D overlay culture in reconstituted basement membrane (rBM). We have applied and cross-validated whole genome microarray (Affymetrix) and digital gene expression (DGE) analyses (Illumina/Solexa) to explore the networks and pathways that underlie DCIS. DGE analysis revealed a broad range of products that are transcribed outside of standard (NCBI 36.3) genes models. These transcripts suggest truncations and changes in anti-sense driven regulatory pathways in DCIS.

3:20 Population Based *in vivo* Biomarker Discovery Using Engineered Human Tumors

Min Wu, Ph.D., Principal Scientist, Translational Research, AVEO Pharmaceuticals, Inc.

Human tumor populations exhibit significant inter-tumor variation, where each tumor harbors a unique set of genetic alterations that impact prognosis and response to treatment. Unfortunately, this variation results in low response rates in the clinic and creates significant challenges for drugs to meet regulatory endpoints. Cancer cell line based xenografts have traditionally been the preclinical model of choice to assess the efficacy of clinical compounds, however, such models exhibit inherent artifacts due to long term *in vitro* culture, and are unable to adequately capture natural variation seen in human tumor populations. To address this challenge, we have created a population based tumor model system based on Human-in-Mouse tissue transgenic human tumors that feature naturally occurring tumor variation akin to that observed in human tumor populations. Each tumor of the population has been comprehensively characterized at the RNA and DNA level, and the population has been adapted to conduct quantitative efficacy studies of anti-cancer agents and combinations, enabling correlations between response and the genetic context of the tumors. This platform enables us to identify and validate biomarkers of therapeutic response in an *in vivo* human tumor system.

1:45 Dessert in the Exhibit Hall



## 10:30 Poster Competition Refreshment Break & Raffles in the Exhibit Hall

### 11:20 Metabolomic Analysis of Prostate Cancer Progression

Arun Sreekumar, Ph.D., Molecular Oncology Program, Genomics/Epigenomics, Medical College of Georgia

Prostate cancer is the second most common cause of cancer-related death in men in the United States and afflicts one out of nine of those over the age of 65. There is an urgent need to develop biomarkers that can supplement PSA and increase its specificity for prostate cancer. The advent of high throughput profiling strategies has allowed scientists to look at global changes in genome, proteome and metabolome. Metabolomics, unlike genomics and proteomics, is a young science that has the potential to radically alter the future of healthcare, drug discovery, and drug delivery. It is the single best window into the cellular state discovered to date. Like the other omics-style sciences, where genomics is best understood as defining the genetic potential, transcriptomics is a window into the future (desired) direction of the cellular activity, and proteomics is a window to the functional potential of the cell; metabolomics, the omics science of metabolism, is the only window into the current and actual state of the cell (or by extension, organism) at a specific point in time.

Recently we have profiled the metabolome in prostate cancer progression using a combination of GC and LC chromatography. Our study quantified the levels of >1000 metabolites across 250 biospecimens. Results of the profiling study revealed elevated levels of sarcosine or N-methyl glycine to be associated with advanced prostate cancer. Importantly components of sarcosine pathway were found to regulate prostate cancer aggressivity. In addition to sarcosine we have defined additional metabolites that are being characterized in the context of prostate cancer progression. Our long term objective is to define a multiplex panel of metabolomic markers for prostate cancer progression.

### 11:45 Circulating Tumor Cells: From Enumeration to Comprehensive Characterization

Nicholas C. Dracopoli, Ph.D., VP, Biomarkers, Centocor R&D, Inc., Johnson & Johnson

Circulating tumor cells (CTC) are very rare and consist of about 1 in 108 or 109 cells in blood drawn from some patients with metastatic cancer. Enumeration of CTCs has been shown to have prognostic value for patients with metastatic breast prostate and colorectal cancer, and is being evaluated to determine if a treatment-related reduction in CTC counts is predictive of therapeutic response. Comprehensive characterization (DNA, RNA and protein) of CTCs will significantly add to the value of CTC enumeration tests, and enable serial monitoring of CTCs for molecular changes occurring during disease progression and as a response to therapy. This presentation will review new approaches for the isolation and characterization of CTCs, and discuss how CTC-derived biomarkers will become a critical factor in the development of personalized treatment strategies in oncology.

### 12:10 PM Progress in Noninvasive Detection of Nucleic Acid Biomarkers

Charles R. Cantor, Ph.D., CSO, Sequenom, Inc.

Procedures have been developed to enhance the collection of RNA and DNA fragments that enter the peripheral circulation as a result of apoptosis. These include optimized methods of recovering small fragments, amplifying them and then detecting and quantifying sequence characteristics by nucleic acid mass spectrometry. The methods show promise in noninvasive prenatal diagnostics, tumor detection and characterization, and infectious disease agent identification. The overall process is considerably more sensitive and precise than commonly used alternatives.

### 12:35 Luncheon Presentation I

Sponsored by  NanoBioDiscovery

#### Enhanced Sensitivity of Biomarker Detection and Identification Using Nano-scale Protein Arrays

Jennifer Ohayon, Ph.D., BioDiscovery Project Leader, NanoInk, Inc.

Nanoscale protein arrays will become an essential technology in the pursuit of personalized medicine. NanoInk has developed several DipPen Nanolithography (DPN) platforms, providing a direct write spotting technique capable of generating sub-micron sized features of biomolecules on solid surfaces. The resultant enhancement in biomarker sensitivity will provide

a more complete understanding of human disease from a systems biology approach.

### 1:05 Luncheon Presentation II (Sponsorship Opportunity Available)

### 1:45 Ice Cream Refreshment Break in the Exhibit Hall

## PLENARY KEYNOTE SESSION

### 2:15 Plenary Keynote Introduction

### 2:25 Plenary Keynote Presentation (See Page 26 for Details)

### 3:05 Refreshment Break in the Exhibit Hall

## NEXTGEN SEQUENCING AS A CLINICAL TOOL

### 3:45 Chairperson's Remarks

German Pihan, M.D., Dpt. of Pathology, Beth Israel Deaconess Medical Center

### 3:50 Enabling Personalized Medicine: The Growing Role of Next Generation Sequencing

German Pihan, M.D., Department of Pathology, Beth Israel Deaconess Medical Center

Ready access to the genome sequence of a patient is arguably the single most important factor in the implementation of personalized medicine. The recent development of massive parallel sequencing technologies promise to make personalized medicine soon a reality. Here I review the technological state-of-the-art as well as the clinical areas where massive parallel sequencing may have the greatest and most immediate impact.

### 4:20 Keynote Presentation

### HLA Typing by High Resolution Technology

Henry A. Erlich, Ph.D., VP, Discovery Research, Human Genetics, Roche Molecular Systems, Inc.

### 4:50 Next Generation Sequencing for Hypertrophic Cardiomyopathy Diagnostics

Karl V. Voelkerding, M.D., Associate Professor of Pathology, University of Utah and Medical Director for Advanced Technology, ARUP Laboratories

Hypertrophic cardiomyopathy is an autosomal dominant disorder of cardiac sarcomere structure and function leading to multiple cardiac conditions. At least 16 genes with over 450 mutations have been implicated in HCM. We have currently designed and tested a next generation sequencing approach for the analysis of this multi-gene disorder and are refining our approach for diagnostic application.

### 5:20 HIV Dynamics Taught by Sequencing

Ramy Arnaout, M.D., DPhil., Associate Director, Clinical Microbiology, BIDMC Staff Pathologist, Department of Pathology, BIDMC and Harvard Medical School, Beth Israel Deaconess Medical Center

High-throughput sequencing platforms provide an approach for detecting rare HIV-1 variants and documenting more fully quasispecies diversity. We applied this technology to understand viral dynamics at the sequence level associated with antiviral treatment failure. Failure was associated with extreme, rapid shifts in population frequencies toward specific resistant forms, and deep sequencing provided a detailed view of the rapid evolutionary impact of selection.

### 5:50 Close of Day

## FRIDAY, FEBRUARY 5

## MICRORNA DIAGNOSTICS FOR CANCER: TRANSLATING INFORMATION TO PRACTICAL USE

### 8:30 AM Chairperson's Opening Remarks

Dalia Cohen, Ph.D., CSO, Rosetta Genomics, Inc.

### 8:35 Keynote Presentation

### Causes and Consequences of microRNA Dysregulation in Cancer

Carlo M. Croce, M.D., Professor, Internal Medicine, College of Medicine & Public Health, The Ohio State University

During the past several years it has become clear that alterations in the expression of microRNA genes contribute to the pathogenesis of most, perhaps all, human malignancies. These alterations can be caused by a variety of mechanisms, including deletions, amplifications or mutations involving microRNA loci, by epigenetic silencing or by dysregulation of transcription factors targeting specific microRNAs. Since malignant cells show dependence on the dysregulated expression of microRNA genes, which in turn control or are controlled by dysregulation of multiple protein coding oncogenes or tumor suppressor genes, these small RNAs provide important opportunities for development of future microRNA based therapies.

## 9:05 microRNA Polymorphisms and the Future of Personalized Medicine

Prasun J. Mishra, Ph.D., Laboratory of Cancer Biology & Genetics, National Cancer Institute, NIH

Referred to as the micromanagers of gene expression, microRNAs are evolutionarily conserved small non-coding RNAs. Polymorphisms in the microRNA pathway can influence gene regulation and are emerging as powerful tools to study the biology of diseases. Detection of microRNA-polymorphisms can potentially improve diagnosis, treatment and prognosis in patients and has profound implications in the fields of pharmacogenomics and personalized medicine.

## 9:30 Living in a Sequen-omics World: Data Integration Issues and Challenges

David Sugarbaker, M.D., Chief, Thoracic Surgery, Brigham and Women's Hospital

DNA sequencing and other "omics" platforms (e.g., mRNA, microRNA, CGH, exon, SNP, and other arrays) have experienced a technological revolution in throughput and scale over the preceding decade that shows no sign of slowing. However, data storage and processing advances have outpaced the ability to fully integrate the resulting massive quantities of data into biologically meaningful and predictive models to apply to risk estimation, prevention, and cure of human diseases, most notably cancer. In addition, further technological innovation will continue to drive down costs which will exacerbate the problem in the near-term but over the long-term will bring more resources to bear in solving these issues and challenges. This session will provide a comprehensive view of the sequen-omics landscape and identify the key issues that will need to be addressed in the future for these platforms to positively affect human health.

We performed the first miRNAome-wide evaluation of specific miRNA expression in dried, forensically relevant biological fluids (blood, semen, saliva, vaginal secretions and menstrual blood). A panel of nine differentially expressed miRNAs was identified that permit the identification of the body fluid using 50pg of total RNA. miRNA profiling provides a promising alternative approach to body fluid identification for forensic casework.

## 10:05 10:05 LNA™ based Universal RT microRNA PCR System. A new Generation High Throughput QPCR Platform Optimized for Development microRNA based Molecular Diagnostic Assays on Clinical FFPE and Blood Serum and Plasma

Jacob Ulrik Fog, Ph.D., Scientific Manager, Diagnostic Product Development Division, Exiqon A/S

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Using a Locked Nucleic Acid (LNA™) based miRNA detection technology we have developed a high throughput QPCR system for detection of miRNAs in clinical paraffin-embedded tissue as well as blood derived plasma or serum. The use of the LNA™ bases adds critical specificity and sensitivity creating a more robust system for more rapid assay development in the clinical and diagnostic assay development.

## 10:20 Coffee Break

## 11:00 The Onco-SNP and Cancer Risk: microRNA Binding Site Polymorphisms as Biomarkers

Joanne B. Weidhaas, Ph.D., Assistant Professor, Therapeutic Radiology, Yale University

Since microRNAs are global regulators, small aberrations such as SNPs that disrupt their coding sequences or their target binding sites can alter cellular homeostasis and enhance cancer risk. MicroRNA binding site polymorphisms have turned out to be some of the strongest biomarkers of cancer risk, can act as biomarkers of outcome, and may be future targets for therapy.

## 11:30 Role of microRNA Based Profiling in Determining Tissue of Origin for Carcinoma of Unknown Primary

Gauri Varadhachary, M.D., Associate Professor of Medicine, Department of G.I. Medical Oncology, M.D. Anderson Cancer Center

Carcinoma of unknown primary (CUP) is a heterogeneous disease where a patient presents with metastases without an identifiable primary. As more effective cytotoxic and targeted therapies emerge for additional known cancers, accurate identification of CUP subtypes will become increasingly important to the appropriate care of these patients. MicroRNA expression profiling is an emerging tool to help with identification of tissue of origin in patients with CUP.

## 12:00 PM Luncheon Presentation (Sponsorship Opportunity Available) or Lunch on Your Own

### INNOVATIVE DIAGNOSTICS FOR PERSONALIZED MEDICINE

## 1:00 Chairperson's Remarks

Kewal K. Jain, M.D., Professor, CEO, Jain PharmaBiotech

## 1:05 Introduction to the Technologies and their Significance/ Relevance for Personalized Medicine

Kewal K. Jain, M.D., Professor, CEO, Jain PharmaBiotech

## 1:35 A Low Cost Instrument for Microbead-Based Quantitative End-Point PCR and DNA Sequencing

Vera Gorfinkel, Ph.D., Associate Professor, Stony Brook University

We present a novel, low cost instrument which performs microbead-based quantitative end-point PCR and CE based DNA sequencing. The instrument employs ultra fast, single photon sensitive detection of fluorescent signals in capillaries and operates as a module of the hardware/software suite GENOMETRICA - a novel, universal technology platform for molecular biology and medicine.

## 2:05 CNV Studies in Autism and other Neurological Disorders

Jim Chinitz, Chief Executive Officer, Population Diagnostics, Inc.

Historically, patients having a common complex disease have been lumped together and considered homogeneous according to phenotype. Population Diagnostics ("PDx") has led a paradigm shift where appreciation is gaining for the heterogeneity of common disease which is likely caused by highly penetrant rare variants which are multi-genic and independently capable of generating the common phenotype. It is necessary to dissect phenotypes into genotypic differences to understand common disease and to personalize medicine. Beyond SNPs, there is a surprising abundance of structural variation in the genome called Copy Number Variants (CNVs), much of it occurring de novo. Recent studies have revealed "causative" rare CNV associations in autism, schizophrenia and ALS. In these models, the metrics that define the level of clinical relevance (i.e. odds ratios) of the rare variants is unprecedented, making them ideal candidates as novel biomarkers for predictive tests and beacons for molecular pathways. PDx is discovering and using a new standard of "causative" biomarkers and is paving the way for a next generation of diagnostic, personalized medicine and drug discovery applications.

## 2:35 Blood-based Diagnostics of Brain Injuries

Uwe R. Müller, Ph.D., VP, Product Development, Banyan Biomarkers, Inc.

Currently no FDA cleared lab tests exist for TBI and the diagnosis is based on complicated and expensive neurological and radio-imaging tests. Banyan has developed novel biomarkers for detection of TBI in the blood of patients within 2 hours of injury. We will present the results of our ongoing clinical studies, and our progress in the development of appropriate assay systems.

## 3:05 Close of Conference

# Cambridge Healthtech Institute's Third Annual Cancer Molecular Markers

**WEDNESDAY, FEBRUARY 3**

**7:00 AM Registration and Morning Coffee**

**8:00 Plenary Keynote Session** (See Page 26 for Details)

**9:40 Grand Opening Refreshment Break in the Exhibit Hall**

## KEYNOTE PRESENTATIONS

**11:00 Chairperson's Remarks**

*Michael Liebman, Ph.D., Managing Director, Strategic Medicine, Inc.*

**11:10 Personalizing Medicine: It's a System-Based Challenge**

*Franklyn G. Prendergast, M.D., Ph.D., Professor, Pharmacology, Biochemistry & Molecular Biology, Director, Center for Personalized Medicine, Mayo Clinic*

**11:40 Genomic Strategies for Personalized Cancer Treatment**

*Joseph R. Nevins, Ph.D., Barbara Levine Professor, Duke University*

We have made use of expression profiling to develop signatures of oncogenic pathway deregulation that can then be used to profile the state of these pathways within populations of tumors. In addition, the pathway signatures also link the patterns of pathway activation with therapeutics since we have shown that predicting the activation of a pathway also predicts sensitivity to drugs that target the pathway. We have extended this concept to develop more refined signatures that can dissect the complexities of many of the known signaling pathways, providing a more precise capacity to probe the activity or deregulation of the pathway and linking to a broader array of therapeutics.

**12:10 PM Panel: Impact of Personalized Medicine on Oncology Drugs and Treatment**

*Additional Panelist: Mike Boswood, President, CEO, Thomson Reuters*

**12:40 Luncheon Presentation** (Sponsorship Opportunity Available) **or Lunch on Your Own**

**1:45 Dessert in the Exhibit Hall**

## PERSONALIZING THERAPY: TISSUE BIOMARKERS

**2:15 Chairperson's Remarks**

*Tracey Colpitts, Ph.D., Manager, Abbott Molecular*

**2:20 Relating Biomarkers to Efficacy: The Efficacy Curve**

*Tracey Colpitts, Ph.D., Manager, Abbott Molecular*

A method of predicting response in a subgroup defined by a biomarker will be discussed and demonstrated using data from therapeutic trials involving EGFR inhibitors in lung, colon, and breast cancer. Biomarkers that aid in selecting subgroups of patients of response were analyzed and compared. Striking similarities between the different cancers, therapies, and subgroups reveals a relationship between biomarkers and efficacy, which is visualized in the efficacy curve.

**2:50 Network and Pathway Analysis of a Novel 3D Breast Carcinoma Model by Both Digital Gene Expression (DGE) and Whole Genome Array Analyses**

*Ray Mattingly, Ph.D., Associate Professor, Pharmacology, Wayne State University*

We have developed a tractable, *in vitro* model of ductal carcinoma *in situ* (DCIS) based on 3D overlay culture in reconstituted basement membrane (rBM). We have applied and cross-validated whole genome microarray (Affymetrix) and digital gene expression (DGE) analyses (Illumina/Solexa) to explore the networks and pathways that underlie DCIS. DGE analysis revealed a broad range of products that are transcribed outside of standard (NCBI 36.3) genes models. These transcripts suggest truncations and changes in anti-sense driven regulatory pathways in DCIS.

**3:20 Population Based *in vivo* Biomarker Discovery Using Engineered Human Tumors**

*Min Wu, Ph.D., Principal Scientist, Translational Research, AVEO Pharmaceuticals, Inc.*

Human tumor populations exhibit significant inter-tumor variation, where each tumor harbors a unique set of genetic alterations that impact prognosis and response to treatment. Unfortunately, this variation results in low response rates in the clinic and creates significant challenges for drugs to meet regulatory endpoints. Cancer cell line based xenografts have traditionally been the preclinical model of choice to assess the efficacy of clinical compounds, however, such models exhibit inherent artifacts due to long term *in vitro* culture, and are unable to adequately capture natural variation seen in human tumor populations. To address this challenge, we have created a population based tumor model system based on Human-in-Mouse tissue transgenic human tumors that feature naturally occurring tumor variation akin to that observed in human tumor populations. Each tumor of the population has been comprehensively characterized at the RNA and DNA level, and the population has been adapted to conduct quantitative efficacy studies of anti-cancer agents and combinations, enabling correlations between response and the genetic context of the tumors. This platform enables us to identify and validate biomarkers of therapeutic response in an *in vivo* human tumor system.

**3:50 Sponsored Presentation** (Sponsorship Opportunity Available)

**4:20 Reception in the Exhibit Hall** (Sponsorship Available)

**5:20 BREAK-OUT DISCUSSIONS in the Exhibit Hall**

**What is the Forecast for Epigenetics and microRNA?**

*Moderator: Enal Razvi, Ph.D., System Biosciences SBI*

- Status of the microRNA and epigenetics markets
- The research market for microRNA and epigenetics: growth and evolution
- Diagnostics and therapeutics development based on microRNA and epigenetic signatures
- Current challenges and opportunities in these spaces

**Challenges to Whole Genome Sequencing**

*Moderator: s Ng, Ph.D., Assistant Professor, Genomic Medicine, J Craig Venter Institute*

- Challenges to whole-genome sequencing
- Identifying *de novo* and re-current mutations in cancer
- Addressing tumor heterogeneity
- How can we move from characterizing gene variation to utilizing the whole genome
- Sequencing tumors rather than tumor cell lines
- The Complex genomic structure of tumor cells: *de novo* assembly or strategy to detect structural variants

**Are there Cancers of Unknown Primary Tumors?**

*Moderator: Dalia Cohen, Ph.D., Chief Scientific Officer, Rosetta Genomics, Inc.*

- Debate over cancers of unknown primary tumors (CUP)
- Methods to detect CUPs
- Consequences of detection of primary

**Gene Signatures in Cancer Diagnostics**

*Co-Moderators: Gary Geiss, Ph.D., Principal Scientist, NanoString Technologies and David Kern, MBA, Director, MyRaQa*

- Developing a gene signature
- Validation of gene signatures
- Regulatory considerations for gene signature diagnostics

**Systems Chemical Biology-A New Paradigm**

*Moderator: Ally Perlina, Senior Application Scientist, GeneGo Inc.*

- Utilizing tools for drug repositioning
- Understanding side effects
- Understanding the mechanisms of action for drugs
- Networkable compounds

**6:20 Close of Day**

## PERSONALIZING THERAPY: SERUM BIOMARKERS

## 8:25 AM Chairperson's Remarks

Josip Blonder, M.D., Sr Research Scientist; Head, Quantitative Proteomics, NCI Frederick

## 8:30 Personalized Oncoproteomics for Cancer Biomarker

## Discovery: Application to Renal Cell Carcinoma

Josip Blonder, M.D., Sr Research Scientist; Head, Quantitative Proteomics, NCI Frederick

Discovery of diagnostic, therapeutic and prognostic markers is central to personalized treatment of cancer. Thus, proteomic approaches capable of characterizing the patient's tumor phenotype using clinically relevant specimens are critically needed. A method that relies on tissue-directed oncoproteomics is described and applied for cancer biomarker discovery in the plasma of a patient diagnosed with renal cell carcinoma.

## 9:00 Protein Quantification Through Targeted Mass Spectrometry: The Way Out of Biomarker Purgatory?

Steven A. Carr, Ph.D., Director, Proteomics, The Broad Institute of MIT and Harvard

Immunoassays are widely used to measure protein biomarkers in patient blood, but useful antibody reagents do not exist for the vast majority of proteins. We are addressing this serious barrier by developing targeted assay methods employing mass spectrometry to screen and quantify low abundance proteins in plasma. This presentation will focus on the latest developments and applications of these technologies.

## 9:30 Mass Spec for Prostate Biomarkers, Assessing Aggressive vs. Non-Aggressive Prostate Cancer

Jianfeng Xu, M.D., Ph.D., Professor, Wake Forest University School of Medicine

Three types of prostate cancer related genetic variants have been found from genome-wide association studies, including those associated with overall prostate cancer risk, aggressive prostate cancer risk, and higher baseline PSA levels. These genetic variants may have potential clinical utility. However, further studies are needed to assess their clinical validity and clinical utility.

## 10:00 A Novel Tool for Non-Invasive Disease Detection

Jack Leonard, Ph.D., Vice President of Technology

Commercialization, febit Inc.

We developed a novel non-invasive diagnostic assay based on microRNAs. Our Biomarker Signature assay has shown an outstanding performance for the integrative detection of a broad panel of diseases and is well suited for high sample throughput at low cost since for each test less than one minute hands-on time is required. Moreover, our approach stands-out by high reproducibility and sensitivity while test-to-test variations are minimal.

## 10:30 Poster Competition Refreshment Break &amp; Raffles in the Exhibit Hall

## 11:20 Metabolomic Analysis of Prostate Cancer Progression

Arun Sreekumar, Ph.D., Molecular Oncology Program, Medical College of Georgia

Prostate cancer is the second most common cause of cancer-related death in men in the United States and afflicts one out of nine of those over the age of 65. There is an urgent need to develop biomarkers that can supplement PSA and increase its specificity for prostate cancer. The advent of high throughput profiling strategies has allowed scientists to look at global changes in genome, proteome and metabolome. Metabolomics, unlike genomics and proteomics, is a young science that has the potential to radically alter the future of healthcare, drug discovery, and drug delivery. It is the single best window into the cellular state discovered to date. Like the other omics-style sciences, where genomics is best understood as defining the genetic potential, transcriptomics is a window into the future (desired) direction of the cellular activity, and proteomics is a window to the functional potential of the cell; metabolomics, the omics science of metabolism, is the only window into the current and actual state of the cell (or by extension, organism) at a specific point in time.

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Charles R. Cantor, Ph.D., CSO, Sequenom, Inc.

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## 12:35 Luncheon Presentation (Sponsorship Opportunity Available) or Lunch on Your Own

## 1:45 Ice Cream Refreshment Break in the Exhibit Hall

## PLENARY KEYNOTE SESSION

## 2:15 Plenary Keynote Introduction

## 2:25 Plenary Keynote Presentation (See Page 26 for Details)

## 3:05 Refreshment Break in the Exhibit Hall

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## 3:45 Chairperson's Remarks

## 3:50 Enabling Personalized Medicine: The Growing Role of Next Generation Sequencing

German Pihan, M.D., Department of Pathology, Beth Israel Deaconess Medical Center

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## 4:20 Keynote Presentation

## HLA Typing by High Resolution Technology

Henry A. Erlich, Ph.D., VP, Discovery Research, Human Genetics, Roche Molecular Systems, Inc.

## 4:50 Next Generation Sequencing for Hypertrophic Cardiomyopathy Diagnostics

Karl V. Voelkerding, M.D., Associate Professor of Pathology, University of Utah and Medical Director for Advanced Technology, ARUP Laboratories

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Fax: 781-972-5425



## 5:20 HIV Dynamics Taught by Sequencing

Ramy Arnaout, M.D., DPhil., Associate Director, Clinical Microbiology, BIDMC Staff Pathologist, Department of Pathology, BIDMC and Harvard Medical School, Beth Israel Deaconess Medical Center  
High-throughput sequencing platforms provide an approach for detecting rare HIV-1 variants and documenting more fully quasispecies diversity. We applied this technology to understand viral dynamics at the sequence level associated with antiviral treatment failure. Failure was associated with extreme, rapid shifts in population frequencies toward specific resistant forms, and deep sequencing provided a detailed view of the rapid evolutionary impact of selection.

## 5:50 Close of Day

### FRIDAY, FEBRUARY 5

## MICRORNA DIAGNOSTICS FOR CANCER: TRANSLATING INFORMATION TO PRACTICAL USE

### 8:30 AM Chairperson's Opening Remarks

Dalia Cohen, Ph.D., Chief Scientific Officer, Rosetta Genomics, Inc.

### 8:35 Keynote Presentation

#### Causes and Consequences of microRNA Dysregulation in Cancer

Carlo M. Croce, M.D., Professor, Internal Medicine, College of Medicine & Public Health, The Ohio State University

During the past several years it has become clear that alterations in the expression of microRNA genes contribute to the pathogenesis of most, perhaps all, human malignancies. These alterations can be caused by a variety of mechanisms, including deletions, amplifications or mutations involving microRNA loci, by epigenetic silencing or by dysregulation of transcription factors targeting specific microRNAs. Since malignant cells show dependence on the dysregulated expression of microRNA genes, which in turn control or are controlled by dysregulation of multiple protein coding oncogenes or tumor suppressor genes, these small RNAs provide important opportunities for development of future microRNA based therapies.

### 9:05 microRNA Polymorphisms and the Future of Personalized Medicine

Prasun J. Mishra, Ph.D., Laboratory of Cancer Biology & Genetics, NCI, NIH

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David Sugarbaker, M.D., Chief, Thoracic Surgery, Brigham and Women's Hospital

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### 10:05 Sponsored Presentation (Sponsorship Opportunity Available)

### 10:20 Coffee Break

### 11:00 The Onco-SNP and Cancer Risk: microRNA Binding Site Polymorphisms as Biomarkers

Joanne B. Weidhaas, Ph.D., Assistant Professor, Yale University

Since microRNAs are global regulators, small aberrations such as SNPs that disrupt their coding sequences or their target binding sites can alter cellular homeostasis and enhance cancer risk. MicroRNA binding site polymorphisms have turned out to be some of the strongest biomarkers of cancer risk, can act as biomarkers of outcome, and may be future targets for therapy.

## 11:30 Role of microRNA Based Profiling in Determining

### Tissue of Origin for Carcinoma of Unknown Primary

Gauri Varadhachary, M.D., Associate Professor of Medicine, Department of G.I. Medical Oncology, M.D. Anderson Cancer Center  
Carcinoma of unknown primary (CUP) is a heterogeneous disease where a patient presents with metastases without an identifiable primary. As more effective cytotoxic and targeted therapies emerge for additional known cancers, accurate identification of CUP subtypes will become increasingly important to the appropriate care of these patients. MicroRNA expression profiling is an emerging tool to help with identification of tissue of origin in patients with CUP.

## 12:00 PM Luncheon Presentation (Sponsorship Opportunity

Available) or Lunch on Your Own

## TARGETING CANCER STEM CELLS

### 1:00 Chairperson's Remarks

#### 1:05 Impact of Antibodies on Cancer Stem Cells: Discovering Underlying Pathways Essential to Cancer Stem Cell Biology

Tim Hoey, Ph.D., VP, Cancer Biology, OncoMed Pharmaceuticals, Inc.  
Cancer stem cells are thought to mediate tumor initiation, metastasis, and recurrence. We have isolated and characterized CSCs from a variety of major tumor types and have found that these cells are preferentially resistant to many current therapies. As part of our effort to develop novel agents targeting CSCs, we have developed an anti-DLL4 antibody that blocks Notch signaling. Anti-DLL4 inhibits tumor growth through multiple mechanisms including a reduction in CSC frequency.

#### 1:35 Understanding Tumor Cell Heterogeneity in NSCLC:

##### Contributions to Resistance and Relapse

Erica L. Jackson, Ph.D., Scientist, Genentech, Inc.

Tumors are made up of a heterogeneous mixture of cell types and it is possible that distinct cell populations play unique roles in tumorigenesis. We are studying functionally defined cell populations to determine what distinguishes chemo-resistant cells from bulk tumor cells.

#### 2:05 ABC Transporters' Role in Cancer Stem Cell Drug Resistance

Muhammad Al-Hajj, Ph.D., Director, Stem Cell Discovery Unit, GlaxoSmithKline

One of the mechanisms by which residual disease become chemo-resistant is via the decreased efficiency of chemo-therapeutics through the action of ATP-binding cassette (ABC) proteins that are variably expressed by the tumor cells and tend to be up-regulated in some cancer stem cells. The clinical relevance of the ABC transporters in the context of cancer stem cells is paramount and their application requires better understanding of the role individual transporters play in the mechanism and the development of more specific inhibitors with minimal off target effects. Here we'll discuss the role of two specific transporters in pancreatic and colon cancer stem cells and their value as therapeutic targets.

#### 2:35 New Visions of Cancer Therapy through the Prism of the Cancer Stem Cell Hypothesis

Justin D. Lathia, Ph.D., Research Associate, Department of Stem Cell Biology and Regenerative Medicine, Lerner Research Institute, Cleveland Clinic Foundation

The failure of conventional therapies to fundamentally alter the survival of advanced and metastatic cancers has many causes but one appears to be the striking cellular heterogeneity in most cancers. The cancer stem cell hypothesis posits that tumors contain a cellular hierarchy of differentiation and tumor propagation potential. As studies have demonstrated that cancer stem cells display therapeutic resistance, angiogenic potential, and a propensity towards invasion/metastasis, the identification of signaling pathways and molecular targets in cancer stem cells may yield improved cancer therapies.

### 3:05 Close of Conference

# Cambridge Healthtech Institute's Seventh Annual Mastering Medicinal Chemistry

The Senior Level  
Chemist Event

WEDNESDAY, FEBRUARY 3

7:00 AM Registration and Morning Coffee

8:00 Plenary Keynote Presentations (See Page 26 for Details)

9:40 Grand Opening Refreshment Break in the Exhibit Hall

## KEYNOTE PRESENTATIONS: RECENT APPROVALS AND CLINICAL CANDIDATES

11:00 Chairperson's Remarks

Hing Sham, Ph.D., Senior Vice President, Chemical Sciences, Elan Pharmaceuticals

11:10 Recent Approval: Mozobil from Development to Approval

Renato Skerlj, Ph.D., VP, Medicinal Chemistry, Genzyme Corporation Drug and Biomaterial R&D

Mozobil™ (plerixafor injection), a first in class small molecule antagonist of the chemokine receptor CXCR4, was granted marketing approval by the FDA in December 2008 and indicated for the mobilization of hematopoietic stem cells to the bloodstream for collection and subsequent autologous transplantation in patients with non-Hodgkin's lymphoma and multiple myeloma.

11:40 Discovery of a State-Dependent Cav2.2 Blocker for the Treatment of Chronic Pain

Scott B. Hoyt, Ph.D., Research Fellow, Department of Basic Chemistry, Merck Research Laboratories  
Voltage-gated Cav2.2 calcium channels control the release of neurotransmitter at presynaptic terminals, and thus play a critical role in pain signaling. The state-independent Cav2.2 blocker ziconotide, a peptide that must be administered via intrathecal injection, has demonstrated clinical efficacy in the treatment of severe chronic pain. State-dependent Cav2.2 blockers may likewise provide clinical pain relief without adversely affecting other nerve functions.

12:10 PM Discovery of Lorcaserin: A Selective 5-HT2C Agonist for the Treatment of Obesity

Brian Smith, Ph.D., Director, Medicinal Chemistry, Arena Pharmaceuticals, Inc.  
Compelling evidence suggests that drugs which activate the 5-HT2C receptor cause weight loss and thus have potential as anti-obesity agents. Because serotonin elicits a number of biological responses through modulation of other 5HT receptors, selectivity has been a critical challenge. This presentation outlines events, challenges and achievements that led to the discovery and development of lorcaserin.

12:40 Luncheon Presentation (Sponsorship Opportunity Available) or Lunch on Your Own

1:45 Dessert in the Exhibit Hall

## CASE STUDIES FROM CHEMISTRY TO THE CLINIC

2:15 Chairperson's Remarks

2:20 Discovery of SCH 530348 – A Thrombin Receptor Antagonist with Potent Antiplatelet Effects

Samuel Chackalamannil, Ph.D., Distinguished Research Fellow, Discovery Research, Schering-Plough Research Institute

SCH 530348, a himbacine-based thrombin receptor antagonist (TRA), is currently undergoing Phase-III clinical studies for acute coronary syndrome and secondary prevention of cardiovascular events in high risk patients. In a Phase-II clinical study in PCI patients, SCH 530348 showed no statistically significant increase in major or minor bleeding when added to standard of care, and showed a non-statistically significant reduction in major adverse cardiac events, including periprocedural myocardial infarction.

2:50 Macrocyclic-based Drug Discovery: The Ulimorelin Story

Helmut Thomas, Ph.D., Senior Vice President, Research & Preclinical Development,

Tranzyme Pharma, Inc.

The discovery and development path leading from a small molecule macrocyclic screening library to the novel ghrelin receptor agonist, ulimorelin (TZP-101), a Phase III product for the treatment of GI hypo-motility disorders, will be presented in detail. The specific attributes of the medicinal chemistry technology (MATCH™) that enabled the rapid progression of these efforts from hit-to-lead-to-clinic will be outlined.

3:20 Executive Panel

Medicinal Chemistry Drivers: Innovation, Technology, Efficiency and Luck

Moderator: Graeme Semple, Ph.D., Vice President, Discovery Chemistry, Arena Pharmaceuticals, Inc.

Panelists:

Michael Henning, Ph.D., Vice Director & Head, Discovery Technologies, F. Hoffmann-La Roche Ltd

Kenneth A. Savin, Ph.D., Manager, Global External Research & Development, Eli Lilly & Co.

Gary W. Small, M.D., Parlow-Solomon Professor on Aging, Professor of Psychiatry & Biobehavioral Sciences, Director, UCLA Center on Aging

3:50 Thermodynamic Sponsored by SCHRÖDINGER

Contributions of Water Molecules to Ligand-Receptor Binding

Christopher Higgs, Ph.D., MRSC, Senior Applications Scientist, Schrödinger, LLC  
Interpreting structure-activity data is often challenging even with the availability of crystal structures. The role of solvent thermodynamics in protein binding sites is often overlooked but can be important in explaining experimental data. Here, we present a statistical thermodynamic approach to the treatment of binding site water molecules and show that hydration site displacement patterns can be used to explain SAR trends, ligand selectivity, and site-directed mutagenesis. Applications of the method to the A2A adenosine receptor, PDZ domains, a broad range of kinases, and other systems of pharmaceutical interest will be discussed.

4:20 Reception in the Exhibit Hall (Sponsorship Available)

5:20 BREAK-OUT DISCUSSIONS in the Exhibit Hall

6:20 Close of Day

THURSDAY, FEBRUARY 4

## FRAGMENT-INSPIRED AND STRUCTURE-GUIDED MEDICINAL CHEMISTRY

8:25 AM Chairperson's Remarks

Charles Reynolds, Ph.D., Research Fellow, Computer Aided Drug Discovery, Johnson & Johnson

8:30 Drug Discovery Facilitated by Fragment Screening Efforts

Michael Henning, Ph.D., Vice Director & Head, Discovery Technologies, F. Hoffmann-La Roche Ltd

Rapid gain in potency of compounds by structure based drug design together with the high sensitivity of biophysical methods like Surface Plasmon Resonance (SPR) enable the use of fragment molecules to guide drug discovery efforts. The lecture will review the fragment screening efforts at Roche and analyze benefits and challenges of the approach from these experiences. Drug targets like  $\beta$ -secretase or chymase are used as case studies.

9:00 Synthesis, *in vitro* and *in vivo* Evaluation of PI3K Inhibitors


Matthew Burger, Ph.D., Research Investigator II, Global Discovery Chemistry, Novartis Institutes for Biomedical Research

Phosphoinositide-3-Kinase (PI3K) is an important oncology target due to the deregulation of its signaling pathway in a wide variety of human cancers. A lead series from a combinatorial library was identified that potently inhibits PI3K. Using SBDD the lead series was optimized to yield PI3K inhibitors with suitable PK properties to establish a PK/PD-efficacy relationship in a mouse A2780 xenograft model.

## 9:20 Design, Synthesis and Optimization 2-aminoquinazolines as PDK1 Inhibitors

Savithri Ramurthy, Ph.D., Research Investigator, GDC/ONC, Novartis Institutes of Biomedical Research, Emeryville, CA

Herein, we describe the use of iterative structure-guided design to discover two sets of leads from the series Quinazolines as PDK1 inhibitors. The *in vitro* and *in vivo* activity of potent PDK1 inhibitors will be discussed along with the medicinal chemistry approaches utilized to optimize the chemical series for kinase selectivity, efflux, and hERG.

9:40 Presentation Sponsored by  **evotec** [www.evotec.com](http://www.evotec.com)

## Fragment Based Drug Discovery at Evotec - Application to the identification of BACE and PDE10a inhibitors

James Madden, Ph.D., Principal Scientist, Evotec (UK) Limited

Evotec's FBDD platform (EVOlutionTM) integrates orthogonal screening technologies, namely; biochemical, NMR and SPR to test fragments in a high throughput, highly sensitive mode. Evotec has successfully applied this technology in a number of programs. This presentation will describe 2 case studies where EVOlutionTM has been used to discover BACE and PDE10a inhibitors.

10:00 Luncheon Presentation Sponsored by  **THOMSON REUTERS**

## The Role of Medicinal Chemistry in Translational Research

Josep Prous, Jr., Ph.D., MBA, Vice President and Chief Scientific Officer, Thomson Reuters Healthcare & Science

The biomedical community has embraced the translational research approach to finding better and safer medicines. However, to meet the promises of this approach, researchers need a knowledge-based methodology in which the constituent disciplines share data appropriately. This talk will show how medicinal chemistry provides a bridge between early biology findings and clinical application of new molecular entities.

## 10:30 Poster Competition Refreshment Break & Raffles in the Exhibit Hall

## 11:30 Computing Specific Residue-ligand Interaction Energies using Quantum Mechanical Energy Decomposition: A Tool for Guiding Drug Design

Charles Reynolds, Ph.D., Research Fellow, Computer Aided Drug Discovery, Johnson & Johnson

Quantum methods are just beginning to find wider application in drug discovery. We have used pair-wise decomposition of protein-ligand interaction energies, computed using the DivCon program, to analyze the interactions that drive potency in a series of protein kinase B inhibitors. These computed interaction energies were used to derive two heat maps: (1) an interaction energy map and (2) an SAR map. These interaction energies, and resulting maps, provide detailed information not otherwise available for identifying the residues in an active site that are most critical for ligand binding.

## 12:00 PM The Emperor's New Crystal: Examples of X-ray Bloopers; a Cautionary Tale

Edward Kesicki, Ph.D., Director, Small Molecule Drug Discovery, Infectious Disease Research Institute

I will give examples of "solved" structures in which incorrect ligands were fitted to the electron density map of a kinase co-crystal, a result of a typographical error in the paperwork sent to the crystallographer. In addition, I will show a class of selective PI3 kinase inhibitors that would have never been discovered using known X-ray crystal structures.

## 12:30 Luncheon Presentation (Sponsorship Opportunity Available) or Lunch on Your Own

## 1:45 Ice Cream Refreshment Break in the Exhibit Hall

## PLENARY KEYNOTE SESSION

### 2:15 Plenary Keynote Introduction

### 2:25 Plenary Keynote Presentation (See Page 26 for Details)

### 3:05 Refreshment Break in the Exhibit Hall

## TARGETS IN HOT PURSUIT I

### 3:45 Chairperson's Remarks

Thomas Högborg, Ph.D., Vice President, 7TM Pharma, Hørsholm, Denmark

### 3:50 Redesign of Arylsulfonamide Gamma Secretase Inhibitors to Achieve Novelty and High *in vivo* Activity

Andrei Konradi, Ph.D., Senior Director, Medicinal Chemistry, Elan Pharmaceuticals

The transformation of known arylsulfonamide Gamma Secretase Inhibitors (GSIs) into Elan's arylsulfonyl pyrazolopiperidine GSIs, using small molecule modeling and pharmacophore hypotheses, will be described. Exploration of analogs to maximize *in vitro* potency, metabolic stability, pharmacokinetics, and *in vivo* activity will be presented. Several novel synthetic methods to prepare the target compounds will be described.

### 4:20 Discovery of the Nedd-8 Activating Enzyme Inhibitor MLN4924

Steve Langston, Ph.D., Senior Scientist, Millennium Pharmaceuticals, the Takeda Oncology Company

The ubiquitin-proteasome system (UPS) is responsible for the regulated degradation of intracellular proteins with important roles in cellular function including cancer cell growth and survival. NEDD8-activating enzyme (NAE) is an essential component UPS that regulates degradation of a subset of proteins upstream of the proteasome. The discovery of MLN4924, a first in class inhibitor of NAE, will be presented.

### 4:50 Discovery of the Hedgehog Inhibitor GDC-0449

Michael Koehler, Ph.D., Scientist, Discovery Chemistry, Genentech, Inc.

### 5:20 Exploration of SAR and optimization of *in silico* derived CRTH2 antagonists

Thomas Högborg, Ph.D., Vice President, 7TM Pharma, Hørsholm, Denmark

Several chemical series of antagonists for the PGD2 receptor CRTH2 were identified from a small focused library originating from a knowledge-based process using physico-genetic relationships and ligand information. The elucidation of SAR by synthesis of smaller libraries and design of specific target structures led to identification of novel druggable chemotypes.

### 5:50 Close of Day

## FRIDAY, FEBRUARY 5

## STRATEGIES FOR EFFECTIVE MEDICINAL CHEMISTRY

### 8:30 AM Chairperson's Opening Remarks

Kenneth A. Savin, Ph.D., Manager, Global External Research & Development, Eli Lilly & Co.

### 8:35 A Paradigm Change in the Application of ADME Resources to Early Lead Generation Activities

Kenneth A. Savin, Ph.D., Manager, Global External Research & Development, Eli Lilly & Co.

With the advent of new technology and processes, there are new possibilities for the application of ADME resources to projects at earlier phases in a project's life-cycle. We have been able to change the way ADME supports projects with the hope of improving our ability to come to decision points earlier in lead generation.

### 9:05 Understanding Structure-Toxicity Relationships as a Guide to Safer Drugs

John C.L. Erve, Ph.D., DABT, Principal Research Scientist II, Drug Safety Metabolism, Wyeth Research

Reactive metabolites are a concern due to their potential role in drug toxicity. Despite our understanding of bioactivation pathways and ability to minimize reactive metabolite formation, toxicity remains a cause of failure during drug development. This talk will review structure-toxicity relationships so that this knowledge can benefit drug discovery.

**9:35 Biotransformation to Enable Chemistry SAR**

Douglas Spracklin, Ph.D., Director, Pharmacokinetics, Dynamics and Metabolism, Pfizer, Inc.  
Biotransformation science has evolved well beyond traditional structural elucidation of metabolites. Contemporary biotransformation data is especially well suited to aid chemistry SAR development, i.e., identifying metabolic hot spots, non-obvious metabolic pathways, potential reactive metabolites, etc. Knowledge around these attributes can be extremely helpful in prioritizing chemical series and selecting individual molecules for development.

**10:05 Sponsored Presentation (Sponsorship Opportunity Available)****10:20 Coffee Break****IMAGING AS AN EXCITING TOOL IN DRUG DISCOVERY**

Chair: Michael A. Letavic, Research Fellow, Neuroscience, Johnson & Johnson Pharmaceutical R&D

**11:00 Imaging Drug Action in the Human Brain**

Joanna Fowler, Ph.D., Senior Chemist, Brookhaven National Laboratory

Radiotracers and drug molecules labeled with short-lived positron emitting isotopes such as carbon-11 (t<sub>1/2</sub>: 20.4 min), fluorine-18 (t<sub>1/2</sub>: 110 min) or nitrogen-13 (t<sub>1/2</sub>: 10 min) are unique scientific tools for measuring biochemical transformations and drug pharmacokinetics and pharmacodynamics in the living human and animal body.

**11:30 Image Analysis Considerations for Preclinical, *in vivo* Medical Imaging**

Matt Silva, Head, Imaging Sciences, Millennium, The Takeda Oncology Company

With the expanding role of preclinical and translational imaging in drug research, it is necessary to consider not only study design and imaging modality but also visualization and image quantification. This presentation will review the role of imaging technologies and show examples of experiments and image analysis procedures, including kinetic analysis of dynamic contrast-enhanced MRI and bone topology analysis from 3D CT data.

**12:00 PM Luncheon Presentation (Sponsorship Opportunity Available) or Lunch on Your Own****TARGETS IN HOT PURSUIT II****1:00 Chairperson's Remarks**

Nick Terrett, Ph.D., Chief Scientific Officer, Ensemble Discovery Corp.

**1:05 Modulators and Consequences of Hsp90 Regulation by Small Molecules**

Brian S. J. Blagg, Ph.D., Associate Professor of Medicinal Chemistry, The University of Kansas; Winner of the 2009 David W. Robertson Award in Medicinal Chemistry

The 90 kDa heat shock proteins (Hsp90) are molecular chaperones required for the refolding of denatured proteins and the maturation of nascent polypeptides into their biologically active, three-dimensional structures. In fact, numerous proteins represented in all six hallmarks of cancer are dependent upon Hsp90 for conformational maturation. Innovative approaches toward C-terminal inhibition of Hsp90 will be discussed.

**1:35 Design and Synthesis of RDEA119, a Potent and Orally Bioavailable MEK Inhibitor**

Jean-Michel Vernier, Ph.D., VP, Chemistry Discovery, Ardea Biosciences

This presentation will discuss the design, synthesis and structural-activity relationship that led to the discovery of RDEA119, a novel highly potent and selective MEK inhibitor currently in Phase I clinical trial. RDEA119 is being developed under a global license agreement with Bayer HealthCare.

**2:05 From a Concept towards a First-In-Class Drug for a Human Amyloid Disease**

Jeffery W. Kelly, Ph.D., Chair, Molecular and Experimental Medicine, Lita Annenberg Hazen Professor of Chemistry, The Skaggs Institute, The Scripps Research Institute

The seminar will cover the twenty-one year adventure from our initial demonstration that rate-limiting transthyretin tetramer dissociation and monomer misfolding was sufficient for transthyretin amyloidogenesis linked to neurodegeneration, to the recent clinical trial results of FoldRx demonstrating that a transthyretin kinetic stabilizer halts neurodegeneration in familial amyloid polyneuropathy. This is the first pharmacologic evidence supporting the validity of the amyloid hypothesis.

**2:35 DNA-Programmed Chemistry Approach to Macrocyclic Lead Compounds**

Nick Terrett, Ph.D., CSO, Ensemble Discovery Corp.

DNA-programmed chemistry is an integrated platform for the synthesis and screening of macrocycles that interact with protein-protein drug discovery targets such as the oncology target, BCL-XL. We have also discovered a series of macrocycles that competitively antagonize the interaction of TNF $\alpha$  with TNF receptors in both biochemical and cell-based assays, and that also have anti-inflammatory activity *in vivo*.

**3:05 Close of Conference****PRIME POSTER POSITION**

- Your Poster will be available to over 3,000 delegates
- You'll automatically be entered into our Poster Competition, where two winners will receive \$500
- \$50 off your registration fee
- Your Poster Abstract will be published on the conference proceedings link
- Your research will be seen by leaders from pharmaceutical, biotech, academic and government institute
- Posters will be on display for two full days



Reserve Your Space By January 13!



Cambridge Healthtech Institute's Second Annual

# Adopting R&D Informatics Systems Data Management, Integration & Knowledge Management

WEDNESDAY, FEBRUARY 3

**7:00 AM Registration and Morning Coffee**

**8:00 Plenary Keynote Session** (See Page 26 for Details)

**9:40 Grand Opening Refreshment Break in the Exhibit Hall**

## EXECUTIVE STRATEGIES—INTEGRATED R&D INFORMATICS

**11:00 Chairperson's Remarks**

David M. Sedlock, Ph.D., Senior Director, Research & Development Systems, Millennium,  
The Takeda Oncology Company

**11:10 Enterprise Scientific Workflow Environment Drives  
Innovation**

Daniel J. Chin, Ph.D., Senior Principal Research Scientist, Roche Palo Alto  
Most informatics investments increase the efficiency of drug discovery. The introduction of an enterprise-wide scientific workflow platform enables research informatics organizations to shift their efforts towards scientific innovation. Researchers apply scientific workflows for *in silico* experimentation and exploration, leading to scientific hypotheses and discoveries. Enterprise environments enable researchers to share and evolve their scientific workflows, further increasing research productivity. Examples of scientific workflows and the setting required to run scientific workflow platforms effectively in pharmaceutical research will be discussed.

**11:40 The Pistoia Alliance, Inc.—A Construct for  
Precompetitive Collaboration**

Chris Waller, Ph.D., Senior Director, Precompetitive Collaborations, Research,  
Development & Medical Informatics, Worldwide Technology, Pfizer, Inc.

The Pistoia Alliance has been established to provide the foundation of data standards, ontologies and associated web-services to enable the Pharmaceutical discovery workflow through common business terms, relationships and processes. Current progress, learnings and how companies, academics and others can participate in this approach will be described.

**12:10 PM Recent Strategies with Cloud, Wikis, Ontologies  
and Open Source Data Standards**

Giles M. Day, Senior Director, BBC Informatics, Pfizer, Inc.

**12:40 Luncheon Presentation I** Sponsored by **Microsoft**

\*By attending Microsoft's Luncheon presentation you are opting to receive further communications from Microsoft.

**Personalized Medicine: The Missing Pieces**

Jim Karkanas, Senior Director of Applied Research & Technology, Microsoft Health  
Solutions Group

Discoveries to make personalized medicine a reality depend on leveraging the "open universe" of life sciences data. To assist investigators with ad hoc questions, hypothesis generation, and validation, investigators must describe and verify systems about the life science universe. This session will introduce a strongly typed repository of linked data that makes it possible to conceive and deliver game-changing therapies.

**1:10pm Luncheon Presentation II** Sponsored by **BioFortis**  
**Empowering Scientists with Hypothesis-Driven  
Data Exploration**

Jian Wang, Ph.D., CEO, BioFortis Inc.

A significant bottleneck on productivity in translational research is the inability for scientists to directly interrogate data by themselves. We present a novel solution & case study to demonstrate how, with the right tools, scientists can be more self-sufficient, efficient and productive, while enabling informatics specialists to focus more on higher value contributions instead of mundane ad hoc data manipulations.

**1:45 Dessert in the Exhibit Hall**

**2:15 Chairperson's Remarks**

David M. Sedlock, Ph.D., Senior Director, Research & Development Systems, Millennium,  
The Takeda Oncology Company

**2:20 Integrated Informatics Systems for R&D**

Vaibhav A. Narayan, Ph.D., Senior Director, Integrative Neurosciences & Biomarkers,  
Johnson & Johnson Pharmaceutical Research & Development

**2:50 Executive Panel with Q&A**

**Are We Integrating the Right Data: Extending Beyond  
Laboratory Data to Decisions Impacting Project Success**

Moderator: David M. Sedlock, Ph.D., Senior Director, Research & Development Systems,  
Millennium, The Takeda Oncology Company

- Data Aggregation vs. Data Integration
- Data Management vs. Knowledge Management
- Integrating Data Management Systems across Multiple Sites
- Effective Data Integration in Translational Medicine Research
- Role of Open Source Technology in Systems Design
- Is There a 'Cloud' in Your Future?
- Barriers Sharing Research and Development Data
- Process Management vs. Technology Considerations when Deploying New Systems

Panelists:

Susie Stephens, Director, Biomedical Informatics, Pharmaceutical Research &  
Development, Johnson & Johnson

Daniel J. Chin, Ph.D., Senior Principal Research Scientist, Roche Palo Alto

Chris Waller, Ph.D., Senior Director, Precompetitive Collaborations, Research,  
Development & Medical Informatics, Worldwide Technology, Pfizer, Inc.

Giles M. Day, Senior Director, BBC Informatics, Pfizer, Inc.

**3:50 Presentation**

Sponsored by  **accelrys**

**Workflow based Enterprise Informatics**

Frank Brown, Ph.D., Vice President & Chief Scientific Officer, Accelrys

Accelrys is producing a new generation of Enterprise Informatics systems for chemical, biological and image data registration and mining. The new generation features workflow driven application logic and business rules, clients that leverage the latest collaborative environments such as Microsoft SharePoint, and novel storage techniques to handle the complexity and diversity of today's data types.

**4:05 Sponsored Presentation** (Sponsorship Opportunity Available)

**4:20 Reception in the Exhibit Hall** (Sponsorship Available)

**5:20 BREAK-OUT DISCUSSIONS** in the Exhibit Hall

**6:20 Close of Day**

THURSDAY, FEBRUARY 4

## DATA INFORMATION AND KNOWLEDGE MANAGEMENT

### 8:25 AM Chairperson's Remarks

Thomas P. Hill, Principal, The Leverage Innovation Group; former Director, Learning and Knowledge Management, Genentech

### 8:30 SparkLab 360 - The Complete System for Managing your Lab Research

Roi Paz, Chief Executive Officer, SparkLix Bio-IT

Electronic lab notebooks (ELNs) and laboratory information management systems (LIMS) are essential tools in lab management. SparkLix – an Innovation Award recipient from the Association for Laboratory Automation – developed SparkLab 360, which integrates features from ELNs and LIMS in one user-friendly system. This presentation focuses on how SparkLab enables design, planning, execution and analysis of the entire research process.

Sponsored by



### 9:00 Knowledge for Strategic Advantage: Accelerating the R&D Cycle

Thomas P. Hill, Principal, The Leverage Innovation Group; former Director, Learning and Knowledge Management, Genentech

This presentation focuses on key elements of knowledge leveraged for strategic advantage in the Life Sciences industry, the key challenge of how to accelerate the R&D process by using collaborative informatics technologies and an examination of specific scientific business solution implementations for results. In addition, the key features of a robust collaborative scientific business solution will be identified.

### 9:30 ASAP-Emphasizing Multidimensional Drug Discovery

W. Patrick Walters, Ph.D., Senior Research Fellow & Group Head, Computational Drug Discovery Technologies, Vertex Pharmaceuticals, Inc.

ASAP is new software platform designed to help drug discovery teams make better decisions. ASAP provides an intuitive overview of the data that also allows scientists to easily “drill down” and examine the details of particular experiments. A combination of “filters” and heat maps allows teams to focus on aspects of the data while remaining aware of the “big picture”.

### 10:00 RiSe Architecture – Architectural Aspects of an Integrated Research Informatics Platform

Ajay Shah, Ph.D., MBA, PMP, Director of Research Informatics, Elan Pharmaceuticals Inc.

Elan and Infosys are building a research data integration platform called RiSe (Research Informatics System at Elan). RiSe enables registration of biological entities, their inventory, associated workflows, and integration with chemical data utilizing a workflow driven, multi-tiered, SOA based architecture built on the Microsoft.NET platform. To maximize extensibility in a research environment, the database combines Entity-Attribute-Value design for flexible definition of entities, efficiency-prioritized OLTP schema for inventory management, and a planned ETL interface to a semantic database.

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### 10:30 Poster Competition Refreshment Break & Raffles in the Exhibit Hall

### 11:30 Agile Software Development: Meeting the Rapidly Changing Needs in Drug Discovery

Man-Ling Lee, Ph.D., Senior Program Analyst, Discovery Chemistry, Small Molecule Drug Discovery, Genentech, Inc.

To support drug discovery project teams meeting their timelines, the CompChem/ChemInformatics Group at Genentech has established an agile approach to satisfy the changing needs. The basis are two flexible software platforms, AEREA (Aestel Scientific Information) and Pipeline Pilot (Acceleris). The presentation will discuss the implementation and impact of two applications: one for lead selection and one for DMPK data analysis.

## INTEGRATIVE DATA MANAGEMENT THROUGH CLOUD, WIKIS, ONTOLOGIES & SEMANTIC WEB



Online: [Tri-Conference.com](http://Tri-Conference.com)



Email: [reg@healthtech.com](mailto:reg@healthtech.com)



Fax: 781-972-5425

### 12:00 PM Application of Translational Informatics in Tailored Therapeutics

Susie Stephens, Director, Biomedical Informatics, Pharmaceutical Research & Development, Johnson & Johnson

### 12:30 Managing Research Portfolios – Can IT help you?

Arvinth Balakrishnan, Vice President Life Sciences, Oracle  
Joe Duncan, Chief Executive Officer, Teranode

Research departments work on hundreds of thousands of molecules, markers and targets. How can we simplify information gathering across your scientific community in order make consistent portfolio management decisions? This talk will focus on using Oracle technologies like semantic web; and how portfolios can be reflected in best in class portfolio management tools.

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### 1:00 Luncheon Presentation

Sponsored by



### SOA-based IT Framework for Life Science Research

David A. Medina, Worldwide Life Science and Pharma Segment Executive, Hewlett-Packard Company

This presentation will present a collaborative platform for bioinformatics used in bioresearch based on a scalable, standards-based, easy-to-deploy, SOA-based architecture. This platform will facilitate the integration of intra-organizational research efforts and enable inter-organizational R&D collaboration. The platform will also enable pharma R&D organizations to effectively access disparate data sources and facilitate the cross-analysis of genomic, proteomic and clinical data.

### 1:45 Ice Cream Refreshment Break in the Exhibit Hall

## PLENARY KEYNOTE SESSION

### 2:15 Plenary Keynote Introduction

### 2:25 Plenary Keynote Presentation (See Page 26 for Details)

### 3:05 Refreshment Break in the Exhibit Hall

## INTEGRATIVE DATA MANAGEMENT THROUGH CLOUD, WIKIS, ONTOLOGIES & SEMANTIC WEB CONT.

### 3:45 Chairperson's Remarks

Susie Stephens, Director, Biomedical Informatics, Pharmaceutical Research & Development, Johnson & Johnson

### 3:50 Data Integration—What's in it for Me?

Randal Chen, Ph.D., Director, Research Informatics, Amgen, South San Francisco

### 4:20 Semantic Web and Cloud Computing for Integrative Data Management and Analysis Infrastructure

Jonas S. Almeida, Ph.D., Abell-Hanger Distinguished Professor, Bioinformatics and Computational Biology, University of Texas, M.D. Anderson Cancer Center

The systems nature of biological processes and the scalability of cloud computing created an irresistible trend towards distribution of both the data and the ecosystem of applications that analyze them. Accordingly, at M.D. Anderson Cancer Center we are exploring the use of semantic web to render distributed infrastructure manageable and its contents safely discoverable. An open-source prototype was developed, see [s3db.org](http://s3db.org).

### 4:50 Collaborative Drug Discovery Humanitarian and Commercial Researcher Network Case Studies

Barry A. Bunin, Ph.D., Chief Executive Officer & President, Collaborative Drug Discovery (CDD), Inc.

Collaborative Drug Discovery (CDD) has created a community based platform that combines traditional drug discovery informatics with Web2.0 features to provide the best of both worlds. Recent efforts to selectively arrest TB in the dormant phase working with leading researchers and mining SAR data will be presented. A global community of leading TB researchers supported by leading foundations will be reviewed. Advances from communities working together on commercial

drug discovery bringing together industry, foundations, and academia will also be emphasized.

## 5:05 Panel: Drug Discovery Collaborations in 2010

Moderator: Barry A. Bunin, Ph.D., Chief Executive Officer & President, Collaborative Drug Discovery (CDD), Inc.

- Biopharmaceutical – CRO collaborations
- Virtual Pharmaceutical collaborations
- PPP (academic-industry-foundation) collaborations

Panelists:

Vaibhav A. Narayan, Ph.D., Senior Director, Integrative Neurosciences & Biomarkers, Johnson & Johnson Pharmaceutical Research & Development  
 Uli Schmitz, Ph.D., Director, Structural Chemistry, Gilead  
 Adam Renslo, Ph.D., Associate Director, Chemistry, Adjunct Assistant Professor, Pharmaceutical Chemistry

5:50 Close of Day

## FRIDAY, FEBRUARY 5

### TRANSLATIONAL INFORMATICS— HOW FAR HAVE WE COME?

#### 8:30 AM Chairperson's Opening Remarks

Pearl S. Huang, Ph.D., Integrator, Oncology Franchise, Research & Early Development, Merck & Co.

#### 8:35 Implementing a Translational Biomarker Strategy to Reduce Attrition in Drug Development

Irina Antonijevic, M.D., Ph.D., Director, Translational Research, Biological Research, Lundbeck Research, Inc. USA

Early efforts towards the discovery of molecular biomarkers for CNS disorders are encouraging. However, confirmation, and ultimately validation of such biomarkers is dependent on state-of-the-art bioinformatics analyses as well as assay development. These prerequisites will ensure identification of biomarkers that are reproducible and hence of clinical relevance.

#### 9:05 High Content Mining of Disease Biomarkers

Jake Chen, Ph.D., Assistant Professor, Informatics & Computer Science, Indiana University School of Informatics; Director, Indiana Center for Systems Biology and Personalized Medicine, Indiana University-Purdue University Indianapolis; Founder, MedeoLinx, Inc.

To facilitate the interpretation of raw Omics data into detailed disease-specific knowledge of candidate biomarkers, we developed a "high-content biomarker mining" software system. The system can help manage and correlate molecular functions, molecular connectivity, biological pathways, and literature information. Its application into the current biomarker development process will help improve the success rate and quality of candidate biomarkers.

#### 9:35 Single Molecule Real Time Biology: New technologies Enabling a More Complete Characterization of Disease Biology

Eric Schadt, Ph.D., Chief Scientific Officer, Pacific Biosciences

While there has been an explosion of technologies that enable more comprehensive characterizations of complex biological processes like common human diseases, we are still unable to glimpse a large enough fraction of the biology of these systems to build models that are predictive enough to achieve clinical utility. However, with a new wave of technologies on the horizon, providing for the capability to examine the activity of single molecules real time, we will soon be capable of generating the right scale and diversity of data (DNA sequence, RNA sequence, real time monitoring of mRNA translation, full characterizations of base modifications in genomes and transcriptomes) at low cost to dramatically enhance the construction of models for common human diseases that achieve clinical utility. I will cover the single molecule real time (SMRT) technologies from Pacific Biosciences and how these technologies will revolutionize our ability to characterize living systems, and then present a number of integrative biology approaches

to taking the types of data SMRT technologies will generate to get at predictive models of disease that can be used to drive the identification and validation of drug targets and biomarkers.

#### 10:05 Sponsored Presentation (Sponsorship Opportunity Available)

#### 10:20 Coffee Break

#### 11:00 Profiling Patients to Drive Biomarker Development

N. R. Nirmala, Ph.D., Director, Biomarker Analysis and Informatics Unit, Translational Sciences, Novartis Institutes of Biomedical Research

Gene expression profiling is one of the key ways in which a genome-wide view of a patient's response to drug treatment can be obtained. Such a molecular level view can provide strategies for customized therapies in many contexts. In this talk, the opportunities and challenges that this technology presents will be discussed with a couple of case studies. Extension of this approach to other technologies will also be presented in the context of biomarker development.

#### 11:30 Panel: Informatics at R&D Interphases

Moderator: Pearl S. Huang, Ph.D., Integrator, Oncology Franchise, Research & Early Development, Merck & Co.

- Linking clinical outcome with molecular data: filling the gaps
- Capturing uniform clinical language for outcomes
- Compatible and user-friendly data systems—can one size fit all?
- Disease cohorts-how many, how big, what is acceptable quality

#### 12:00 PM Luncheon Presentation (Sponsorship Opportunity Available) or Lunch on Your Own

### INTEGRATED GENOMIC, BIOLOGY, AND IMAGE DATA

#### 1:00 Chairperson's Remarks

Ajay Shah, Ph.D., MBA, PMP, Director, Research Informatics, Elan Pharmaceuticals, Inc.

#### 1:05 Image Analysis Considerations for Pre-clinical, *in vivo* Medical Imaging

Matt Silva, Head, Imaging Sciences, Millennium, The Takeda Oncology Company

With the expanding role of preclinical and translational imaging in drug research, it is necessary to consider not only study design and imaging modality but also visualization and image quantification. This presentation will review the role of imaging technologies and show examples of experiments and image analysis procedures, including kinetic analysis of dynamic contrast-enhanced MRI and bone topology analysis from 3D CT data.

#### 1:35 RISE Prowler: A Semantic Web Approach to Integrating External and In-house Biology and Chemistry Information

Ajay Shah, Ph.D., MBA, PMP, Director, Research Informatics, Elan Pharmaceuticals, Inc.

#### 2:05 Development of a Registration System for Biologics in a Collaborative Special Interest Group

Jeremy Packer, Ph.D., Head, Bioinformatics, Abbott

#### 2:35 Development of Combination Therapies for Multiple Sclerosis Using Systems Level Informatics

Frederic S. Young, Ph.D., Chief Scientist, Vicus Therapeutics

We start with a multilevel systems physiology model that combines metabolomic analysis with integrated physiological analysis. The model is used to define a set of systems informatic features of ontogeny, phylogeny, homeostasis, and repair that distinguishes the disease state from homeostasis. We describe our use of this systems informatic signature as an algorithm for the development of combination therapies for multiple sclerosis.

#### 3:05 Close of Conference



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# Cambridge Healthtech Institute's Sixth Annual Cancer Profiling and Pathways: Next Sequence in the War against Cancer

**WEDNESDAY, FEBRUARY 3**

**7:00 AM Registration and Morning Coffee**

**8:00 Plenary Keynote Session** (See Page 26 for Details)

**9:40 Grand Opening Refreshment Break in the Exhibit Hall**  
**KEYNOTE PRESENTATIONS**

**11:00 Chairperson's Remarks**

*Michael Liebman, Ph.D., Managing Director, Strategic Medicine, Inc.*

**11:10 Personalizing Medicine: It's a System-Based Challenge**

*Franklyn G. Prendergast, M.D., Ph.D., Professor, Pharmacology, Biochemistry & Molecular Biology, Director, Center for Personalized Medicine, Mayo Clinic*

**11:40 Genomic Strategies for Personalized Cancer Treatment**

*Joseph R. Nevins, Ph.D., Barbara Levine Professor, Breast Cancer Genomics, Center for Applied Genomics & Technology, Duke University*  
Perhaps the major challenge in developing more effective therapeutic strategies for the treatment of most major cancers is confronting the heterogeneity of the disease, recognizing that most cancers are not one disease but multiple disorders with distinct underlying mechanisms. We have made use of expression profiling to develop signatures of oncogenic pathway deregulation that can then be used to profile the state of these pathways within populations of tumors. In addition, the pathway signatures also link the patterns of pathway activation with therapeutics since we have shown that predicting the activation of a pathway also predicts sensitivity to drugs that target the pathway. We have extended this concept to develop more refined signatures that can dissect the complexities of many of the known signaling pathways, providing a more precise capacity to probe the activity or deregulation of the pathway and linking to a broader array of therapeutics. We suggest that this approach can provide a framework for an overall strategy towards the development of personalized treatment options for the individual patient, including strategies for personalized combination therapy.

**12:10 PM Panel: Impact of Personalized Medicine on Oncology Drugs and Treatment**

*Additional Panelist: Mike Boswood, President, CEO, Thomson Reuters*

- How could information about differences of individuals become a way to improve drug discovery rather than reduce ROI?
- How can you change it to bring in new knowledge?
- How do you change perception of culture?
- Is it purely technology that is needed to solve the problem?

**12:40 Luncheon Presentation I** Sponsored by **nanoString**

**Digital, Multiplexed Measurements of up to 550 mRNAs in Clinically Relevant Sample Types Using the nCounter™ Analysis System**

*Gary Geiss, Ph.D., Principal Scientist, NanoString Technologies, Inc.*

The nCounter Analysis System utilizes color-coded molecular barcodes to digitally count nucleic acid molecules. The system can multiplex 550 targets without enzymatic manipulation. A variety of input samples have been tested, including FFPE and crude cell lysates. The technology has been applied to multi-gene expression cancer signatures and mRNA fusion-transcripts. Products to measure miRNAs and dsDNA are under development.

**1:10-1:40 A Novel Genome-Wide Screening Application Using Pooled Viral miRNA-adapted shRNA (shRNAmir) Libraries**

*Katie Jansen Spayd, Ph.D., Research Scientist, Thermo Fisher Scientific*

Pooled shRNA libraries are powerful genetic discovery tools that allow for high-throughput screening of the entire genome in a cost-effective and less labor-intensive manner. Unlike arrayed shRNA library approaches requiring many multi-well plates, screens using lentiviral shRNA pools can be performed in a single tissue culture plate. Clonal populations of cells expressing an individual shRNA become enriched or depleted in this mixed population in response to a selective pressure. Genes required for cell enrichment or cell depletion can then be deconvoluted by next-generation sequencing or microarrays hybrid-

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ized with barcode sequences corresponding to each shRNA in the pool. Here, we present a novel pooled shRNA screening approach for identifying regulators of endogenous gene expression. Epithelial cell adhesion molecule (EpCAM), a cell surface receptor that is highly expressed in a variety of tumorigenic cells, promotes cell proliferation and tumor formation via transcriptional activation of mitogenic genes. Thus, EpCAM represents a target for the development of new cancer therapeutics. We performed a whole-genome pooled shRNA screen to identify novel regulators of EpCAM expression. OVCAR8 cells were transduced with the Thermo Fisher Scientific Decode™ RNAi Viral shRNAmir Pools. Following puromycin selection, we used magnetic-activated cell sorting (MACS) to separate cells on the basis of EpCAM protein expression. Genomic DNA was isolated from EpCAM+ and EpCAM- cells and the shRNAs enriched or depleted within each population were identified using custom microarrays. The genes targeted by shRNAs enriched in EpCAM- cells were identified as candidate regulators of EpCAM expression. This work demonstrates that pooled shRNA libraries may be used in a variety of novel screening strategies, including the identification of novel regulators of tumor-associated genes.

**1:45 Dessert in the Exhibit Hall**

**1:45 Dessert in the Exhibit Hall**

**WORKING BACKWARDS IN CANCER:  
FROM THE CLINIC TO DISCOVERY**

**2:15 Chairperson's Remarks**

*Michael Liebman, Ph.D., Managing Director, Strategic Medicine, Inc.*

**2:20 Using Drug-Induced Feedback Loops to Identify Indications and Combination Partners**

*Donald Bergstrom, Director, Experimental Medicine Oncology, Merck*  
*James W. Watters, Associate Director, Molecular Profiling Oncology, Merck*

Treatment with molecular targeted agents can result in compensatory feedback regulation as cells respond to inhibition of signaling pathways. We will present clinical evidence that treatment with a small molecule inhibitor of gamma secretase results in pathway modulation and compensatory feedback, and describe pre-clinical experiments designed to leverage this concept for drug response prediction.

**2:50 Genomic Solutions to Diagnostic and Prognostic Clinical Predictions in Head and Neck Cancer**

*Geoffrey Childs, Ph.D., Professor of Pathology, Albert Einstein College of Medicine*

*Richard V. Smith, M.D., FACS, Professor, Vice-Chair, Department of Otorhinolaryngology-Head and Neck Surgery, Montefiore Medical Center and Albert Einstein College of Medicine*

The strategy our group employs is to utilize the data obtained from high throughput assays including gene expression measurements of mRNA and miRNA, global methylation patterns of DNA and global proteomics to develop prognostic and diagnostic signatures to predict outcome, local regional recurrence presence/absence of lymph node metastasis at initial diagnosis and to predict optimal treatment options.

**3:20 Moving Research Closer to the Bedside, *in vitro* and *in vivo* Analyses with Primary Tumors**

*Fred Poordad, M.D., Chief of Hepatology, Liver Disease and Transplant Center, Cedars-Sinai Medical Center, Xin Wei Wang, Ph.D., Senior Investigator Head, Liver Carcinogenesis Section, Laboratory of Human Carcinogenesis, National Cancer Institute, NIH, and Michael R. Briggs, Ph.D., Senior Director, Biology, Vertex Pharmaceuticals, Inc.*

The incidence of Primary Liver Cancer is increasing in the west and constitutes a tremendous burden on world health as the third leading cause of cancer deaths worldwide. The 5 year survival rate is a dismal 11 %, due in large part to late diagnosis and limited treatment options. The etiology of this devastating disease as well as current and proposed new therapies will be discussed. Steps to better diagnose and stratify patients for targeted therapy will be considered as a new and exciting phase of cancer research. Finally, a move toward more relevant research will be presented as an hypothesis that

will be tested in the coming years as more new and current therapies are compared and contrasted to current best practice.

#### 4:05 How do you go from Pathways to Clinical Outcomes?

Aris Persidis, Ph.D., President, Biovista, Inc.



In drug discovery and development what really counts is the clinical outcome, the Benefit/Risk of the drug within the context of its pathway or mechanism of action (MoA). Biovista screens the MoA of any drug or target against the MoA of 8,000 indications and 12,000 adverse events (AEs). This is simultaneous, unbiased indication discovery and AE profiling, and it is unique. It helps to bridge the gap from molecular pathway to clinical outcome in a single step. Case studies in cancer and other diseases will be described.

#### 4:35 Reception in the Exhibit Hall (Sponsorship Available)

#### 5:20 BREAK-OUT DISCUSSIONS in the Exhibit Hall

##### What is the Forecast for Epigenetics and microRNA?

Moderator: Enal Razvi, Ph.D., System Biosciences SBI

- Status of the microRNA and epigenetics markets
- The research market for microRNA and epigenetics: growth and evolution
- Diagnostics and therapeutics development based on microRNA and epigenetic signatures
- Current challenges and opportunities in these spaces

##### Challenges to Whole Genome Sequencing

Moderator: s Ng, Ph.D., Assistant Professor, Genomic Medicine, J Craig Venter Institute

- Challenges to whole-genome sequencing
- Identifying de novo and re-current mutations in cancer
- Addressing tumor heterogeneity
- How can we move from characterizing gene variation to utilizing the whole genome
- Sequencing tumors rather than tumor cell lines
- The Complex genomic structure of tumor cells: de novo assembly or strategy to detect structural variants

##### Are there Cancers of Unknown Primary Tumors?

Moderator: Dalia Cohen, Ph.D., Chief Scientific Officer, Rosetta Genomics, Inc.

- Debate over cancers of unknown primary tumors (CUP)
- Methods to detect CUPs
- Consequences of detection of primary

##### Gene Signatures in Cancer Diagnostics

Co-Moderators: Gary Geiss, Ph.D., Principal Scientist, NanoString Technologies and David Kern, MBA, Director, MyRaQa

- Developing a gene signature
- Validation of gene signatures
- Regulatory considerations for gene signature diagnostics

##### Systems Chemical Biology-A New Paradigm

Moderator: Ally Perlina, Senior Application Scientist, GeneGo Inc.

- Utilizing tools for drug repositioning
- Understanding side effects
- Understanding the mechanisms of action for drugs
- Networkable compounds

#### 6:20 Close of Day

### THURSDAY, FEBRUARY 4

#### REAL EXAMPLES OF INTEGRATING PATHWAY DATA

##### 8:25 AM Chairperson's Remarks

Megan Laurance, Ph.D., Senior Scientist, Ingenuity Systems, Inc.

##### 8:30 Keynote Presentation

Kenneth H. Buetow, Ph.D., Associate Director, Bioinformatics and Information Technology, National Cancer Institute

##### 9:00 Cooperative and Complementary Genetic Selection in Brain Tumors

Markus Bredel, M.D., Ph.D., Director, Northwestern Brain Tumor Institute Research Program, Assistant Professor, Department of Neurological Surgery, Robert H. Lurie Comprehensive Cancer Center, Northwestern University Feinberg School of Medicine, and Lurie Center for Cancer Genetics Research and Center for Genetic Medicine  
Brain tumors are a disease of the genome. These tumors show recurrent patterns of genetic aberrations. Dissecting which genetic events function cooperatively to deregulate principal signaling pathways in brain tumors and which are complementary to such

deregulation will help developing refined therapeutic strategies to treat these complex diseases.

#### 9:30 Expression Based Patient Stratification for Cancer Prognostics

Peter J. van der Spek, Ph.D., Department of Bioinformatics, Erasmus MC - Medical Faculty

Systems biology approaches in life sciences and health open new perspectives for patient stratification. Microarray and next-generation sequencing techniques provide vast volumes of data and detailed information about natural variants vs. mutations in the underlying molecular etiology of the disease. Knowledge bases allow scientists to place their research results in perspective.

#### 10:00 Functional Analysis of Omics Data in Cancer

Yuri Nikolsky, Ph.D., CEO, GeneGo, Inc.

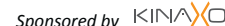
High-throughput assays are indispensable in studies of complex human diseases. Numerous methods have been developed for Omics data analysis. I will describe GeneGo techniques of pathway, network, and interactome analysis, and summarize recent results of our collaborative studies on breast, colorectal, pancreatic, and glioma cancers. I will also describe functional analysis of predictive gene signatures developed for FDA's MAQCII project.



#### 10:15 Cellular Target Profiling and Quantitative Phosphoproteomics Reveal Insight into a Drug's Efficiency and Cellular Mode of Action

Jutta Fritz, Ph.D., Head of Technology, Kinaxo Biotechnologies

System-wide approaches integrating drug target identification and global phosphoproteomics depict a compound's cellular mode of action and its impact on signal transduction. KINAXO's chemical proteomics and global quantitative phosphoproteomics platform revealed Sorafenib's target profile and allowed quantification of phosphorylation patterns in relation to drug administration, thereby facilitating monitoring of the integration of signaling and pointing at additional therapeutic applications.



#### 10:30 Poster Competition Refreshment Break & Raffles in the Exhibit Hall

#### 11:30 microRNA Expression Profiling for the Identification of Forensically Relevant Biological Fluids

Jack Ballantyne, Ph.D., Professor, Department of Chemistry, Associate Director for Research, National Center for Forensic Science, University of Central Florida

We performed the first miRNAome-wide evaluation of specific miRNA expression in dried, forensically relevant biological fluids (blood, semen, saliva, vaginal secretions and menstrual blood). A panel of nine differentially expressed miRNAs was identified that permit the identification of the body fluid using 50pg of total RNA. miRNA profiling provides a promising alternative approach to body fluid identification for forensic casework.

#### 12:00 pm Gene Expression Signatures of Pathway Activity as Biomarkers in Oncology: RAS Pathway Signature

Andrey P. Loboda, Ph.D., Research Fellow, Oncology Molecular Profiling, Merck Research Laboratories

#### 12:30 Luncheon Presentation I Overview of Metabolomics

John Ryals, Ph.D., Chief Executive Officer, Metabolon, Inc.

Metabolomics is the global profiling of biochemicals and metabolites in biological samples and provides a snapshot of the metabolic state of a biological system. As such, it can rapidly characterize and identify metabolic changes caused by drugs, disease, diet or environment effects. This talk provides an overview of metabolomics and the technology requirements for profiling hundreds of biochemicals. The technology platform deployed at Metabolon involves the separation of analytes on three independent analytical platforms (GC-MS, LC-MS/MS(+), LC-MS/MS(-)). Proprietary software processes the mass spectral data and retention times by matching the run data to a database of biochemical standards. This "chemo-centric" approach results in the positive identification of hundreds of biochemicals in a single sample. Through statistical analysis, the significant changes are identified and mapped onto biochemical pathways. Because biochemicals are closely related to biological phenotype, identification of affected pathways not only provides insight into the biological mechanism but uncovers biomarkers useful in diagnosing and monitoring.



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**1:00 Luncheon Presentation II** Sponsored by **BIOBASE**  
**Regulatory Network Analysis of Cancer Gene Expression**  
**Profiles for Target and Biomarker Discovery Using the Explain™**  
**Analysis System**

Raymond DiDonato, Ph.D., Product Manager, BIOBASE Corporation

High-throughput gene expression analysis techniques generate large amounts of data, which pose a particular challenge of transforming expression data into meaningful hypotheses for target discovery and candidate biomarker identification. Traditional approaches for interpreting expression data rely on mapping differentially expressed genes to canonical pathways, biological processes, or disease states. However, understanding the transcriptional regulators and upstream signaling events that lead to differential gene expression can help better identify the molecular mechanisms that influence changes in gene expression profiles, facilitating discovery of targets which themselves are not differentially expressed, but which are key to underlying disease mechanisms. In this session we present a case study in which Explain™, a tool that employs the BIOBASE Knowledge Library™ for promoter and regulatory network analysis, was used to uncover target and biomarker candidates from a cancer gene expression experiment, by identifying upstream signaling molecules likely involved in the underlying pathways leading to the disease state.

**1:45 Ice Cream Refreshment Break in the Exhibit Hall**

**PLENARY KEYNOTE SESSION**

**2:15 Plenary Keynote Introduction**

**2:25 Plenary Keynote Presentation** (See Page 26 for Details)

**3:05 Refreshment Break in the Exhibit Hall**

**SYSTEMS BASED APPROACHES TO CANCER**  
**SEQUENCING: PUTTING TOGETHER A NETWORK OF**  
**CHANGES**

**3:45 Chairperson's Remarks**

Robert L. Strausberg, Ph.D., Deputy Director, J. Craig Venter Institute

**3:50 Whole Genome Sequencing in Cancer**

Gad Getz, Ph.D., Head, Cancer Genome Analysis, The Broad Institute

**4:20 Systematic Discovery of Cancer Gene Fusions using**  
**Paired End Transcriptome Sequencing**

Chandan Kumar, Ph.D., Michigan Center for Translational Pathology, University of Michigan

Gene fusions represent common genetic aberrations in cancers that can serve as specific biomarkers and therapeutic targets. The recent discovery of recurrent gene fusions in prostate and lung cancers portends similar aberrations in other common carcinoma. We employ paired end transcriptome sequencing and customized bioinformatic pipelines to characterize gene fusions and chimeric transcripts in cancer.

**4:50 Mapping Cancer Genomics Data to Pathways**

David Haussler, Ph.D., Professor & Director, Biomolecular Science & Engineering, University of California, Santa Cruz

It is essential, but challenging to interpret cancer genomics data in terms of biological meaningful perturbations of molecular pathways within tumor cells. I will discuss a new Cancer Genomics Browser, on the web at genome-cancer.ucsc.edu, that accomplishes this through large-scale data analysis and probabilistic modeling. This methodology is currently being used in several large-scale cancer studies, including the ISPV breast cancer trial, the TCGA project and by one of the SU2C Dream Teams.

**5:20 Analyzing Coding Variants**

Pauline Ng, Ph.D., Senior Scientist, Human Genomic Medicine, J. Craig Venter Institute

Whole genome and whole exome sequencing are identifying a large number of coding variants. Some of these coding variants may have functional consequences that lead to disease. I will discuss the behavior of coding variants and the webtools we have made to analyze them.

**5:50 Close of Day**

**FRIDAY, FEBRUARY 5**

**MICRORNA DIAGNOSTICS FOR CANCER:**  
**TRANSLATING INFORMATION TO PRACTICAL**  
**USE**

**8:30 AM Chairperson's Opening Remarks**

Dalia Cohen, Ph.D., Chief Scientific Officer, Rosetta Genomics, Inc.

**8:35 Keynote Presentation: Causes and Consequences of**  
**microRNA Dysregulation in Cancer**

Carlo M. Croce, M.D., Professor, Internal Medicine, College of Medicine & Public Health, The Ohio State University

During the past several years it has become clear that alterations in the expression of microRNA genes contribute to the pathogenesis of most, perhaps all, human malignancies. These alterations can be caused by a variety of mechanisms, including deletions, amplifications or mutations involving microRNA loci, by epigenetic silencing or by dysregulation of transcription factors targeting specific microRNAs. Since malignant cells show dependence on the dysregulated expression of microRNA genes, which in turn control or are controlled by dysregulation of multiple protein coding oncogenes or tumor suppressor genes, these small RNAs provide important opportunities for development of future microRNA based therapies.

**9:05 microRNA Polymorphisms and the Future of**  
**Personalized Medicine**

Prasun J. Mishra, Ph.D., Laboratory of Cancer Biology & Genetics, National Cancer Institute, NIH

Referred to as the micromanagers of gene expression, microRNAs are evolutionarily conserved small non-coding RNAs. Polymorphisms in the microRNA pathway can influence gene regulation and are emerging as powerful tools to study the biology of diseases. Detection of microRNA-polymorphisms can potentially improve diagnosis, treatment and prognosis in patients and has profound implications in the fields of pharmacogenomics and personalized medicine.

**9:35 Living in a Sequen-omics World: Data Integration Issues**  
**and Challenges**

Gavin Gordon, Ph.D. Co-Director, Thoracic Surgery Oncology Lab, Brigham & Womens Hospital

DNA sequencing and other "omics" platforms (e.g., mRNA, microRNA, CGH, exon, SNP, and other arrays) have experienced a technological revolution in throughput and scale over the preceding decade that shows no sign of slowing. However, data storage and processing advances have outpaced the ability to fully integrate the resulting massive quantities of data into biologically meaningful and predictive models to apply to risk estimation, prevention, and cure of human diseases, most notably cancer. In addition, further technological innovation will continue to drive down costs which will exacerbate the problem in the near-term but over the long-term will bring more resources to bear in solving these issues and challenges. This session will provide a comprehensive view of the sequen-omics landscape and identify the key issues that will need to be addressed in the future for these platforms to positively affect human health.

**10:05 Sponsored Presentation** (Sponsorship Opportunity Available)

**10:20 Coffee Break**

**11:00 The Onco-SNP and Cancer Risk: microRNA Binding Site**  
**Polymorphisms as Biomarkers**

Joanne B. Weidhaas, Ph.D., Assistant Professor, Therapeutic Radiology, Yale University

Since microRNAs are global regulators, small aberrations such as SNPs that disrupt their coding sequences or their target binding sites can alter cellular homeostasis and enhance cancer risk. MicroRNA binding site polymorphisms have turned out to be some of the strongest biomarkers of cancer risk, can act as biomarkers of outcome, and may be future targets for therapy.

**11:30 Role of microRNA Based Profiling in Determining Tissue**  
**of Origin for Carcinoma of Unknown Primary**

Gauri Varadhachary, M.D., Associate Professor of Medicine, Department of G.I. Medical Oncology, M.D. Anderson Cancer Center  
Carcinoma of unknown primary (CUP) is a heterogeneous disease where a patient presents with metastases without an identifiable primary. As more effective cytotoxic and targeted therapies emerge for additional known cancers, accurate identification of CUP subtypes will become increasingly important to the appropriate care of these patients. MicroRNA expression profiling is an emerging tool to help with identification of tissue of origin in patients with CUP.

**12:00 PM Luncheon Presentation** (Sponsorship Opportunity Available)  
**or Lunch on Your Own**

## TARGETING CANCER STEM CELLS

### 1:00 Chairperson's Remarks

*Tim Hoey, Ph.D., VP, Cancer Biology, OncoMed Pharmaceuticals, Inc.*

### 1:05 Impact of Antibodies on Cancer Stem Cells: Discovering Underlying Pathways Essential to Cancer Stem Cell Biology

*Tim Hoey, Ph.D., VP, Cancer Biology, OncoMed Pharmaceuticals, Inc.*

Cancer stem cells are thought to mediate tumor initiation, metastasis, and recurrence. We have isolated and characterized CSCs from a variety of major tumor types and have found that these cells are preferentially resistant to many current therapies. As part of our effort to develop novel agents targeting CSCs, we have developed an anti-DLL4 antibody that blocks Notch signaling. Anti-DLL4 inhibits tumor growth through multiple mechanisms including a reduction in CSC frequency.

### 1:35 Understanding Tumor Cell Heterogeneity in NSCLC: Contributions to Resistance and Relapse

*Erica L. Jackson, Ph.D., Scientist, Department of Tumor Biology and Angiogenesis, Genentech, Inc.*

Tumors are made up of a heterogeneous mixture of cell types and it is possible that distinct cell populations play unique roles in tumorigenesis. We are studying functionally defined cell populations to determine what distinguishes chemo-resistant cells from bulk tumor cells.

### 2:05 New Visions of Cancer Therapy through the Prism of the Cancer Stem Cell Hypothesis

*Justin D. Lathia, Ph.D., Research Associate, Department of Stem Cell Biology and Regenerative Medicine, Lerner Research Institute, Cleveland Clinic Foundation*

The failure of conventional therapies to fundamentally alter the survival of advanced and metastatic cancers has many causes but one appears to be the striking cellular heterogeneity in most cancers. The cancer stem cell hypothesis posits that tumors contain a cellular hierarchy of differentiation and tumor propagation potential. As studies have demonstrated that cancer stem cells display therapeutic resistance, angiogenic potential, and a propensity towards invasion/metastasis, the identification of signaling pathways and molecular targets in cancer stem cells may yield improved cancer therapies.

### 2:35 Close of Conference



# Cambridge Healthtech Institute's Fifth Annual Stem Cells Shaping the Future of Regenerative Medicine

**WEDNESDAY, FEBRUARY 3**

**7:00 AM Registration and Morning Coffee**

**8:00 Plenary Keynote Session** (See Page 26 for Details)

**9:40 Grand Opening Refreshment Break in the Exhibit Hall**

## ADVANCING REGENERATIVE MEDICINE

Addressing the numerous topics central to the theme of regenerative medicine, this meeting assembles prominent researchers, clinicians, businessmen, and regulators who not only are at the cutting edge of their respective fields, but also represent wide areas of expertise. The cross-fertilization of the information presented in the area of stem cell research engineers brainstorming and provides a forum for discussion to enable the rapidly expanding therapeutic potential of regenerative medicine.

**11:00 Chairperson's Remarks**

*Dawn Driscoll, MBA, Ph.D., Principal, DCI*

**11:10 Keynote Presentation**

**Stem Cells: Moving from Discovery Towards the Clinic**

*Alan Trounson, Ph.D., President, California Institute of Regenerative Medicine*

**11:55 Featured Presentation**

**Diabetes Under Control**

*Andrew Rakeman, Ph.D., Scientific Program Manager, Regeneration, Juvenile Diabetes Research Foundation*

Diabetes treatments necessitate the replacement or regeneration of pancreatic beta cells to improve glucose control and avoid serious side effects. Replacement of beta cells has attracted considerable attention with the use of cadaveric islets, pig islets and a variety of adult stem cells. It appears that the best source to date is cells obtained from human embryonic stem cells and there is hope that iPS cells may one day also be an appropriate source. More recently, interest has focused on regenerating the pancreas as a result of access to human progenitors as well as a better understanding of cell proliferation and neogenesis will accelerate the development of beta cell regenerative medicines.

**12:40 PM Luncheon Presentation** (Sponsorship Opportunity Available) **or Lunch on Your Own**

**1:45 Dessert in the Exhibit Hall**

## CONSIDERATIONS FOR ADVANCING REGENERATIVE MEDICINE INTO THE CLINIC

**2:15 Chairperson's Remarks**

*Dawn Driscoll, MBA, Ph.D., Principal, DCI Biotech*

**2:20 hESC-Derived Oligodendrocyte Progenitor Cells-GRNOPC1 for Acute Spinal Cord Injury**

*Edward Wirth III, M.D., Ph.D., Medical Director, Geron Corporation*

**2:50 From Tissue Engineering to Regenerative Medicine: An Evolution in Understanding**

*Damien Bates, M.D., Ph.D., Chief Medical Officer, Organogenesis, Inc.*

**3:20 MSC-Derived SB623 Cells for Stable Stroke**

*Casey Case, Ph.D., Vice President of Research, SanBio, Inc.*  
SB623 cells are derived from bone marrow stromal cells (MSCs). They have shown great potential in models of CNS regeneration. The cells are used allogeneically and implanted directly at the site of injury. Our first clinical application will be in stable ischemic stroke patients. Models of efficacy and safety will be discussed as will issues pertaining to manufacturing and clinical plans.

**3:50 Presentation Sponsored by** **Thermo**  
SCIENTIFIC

**4:20 Reception in the Exhibit Hall** (Sponsorship Available)

**5:20 BREAK-OUT DISCUSSIONS in the Exhibit Hall**  
**Forecasting Cell Therapy Sales - How and Why**

*Host: Dawn Driscoll, MBA, Ph.D., Principal, DCI Biotech*

Investors, Manufacturing, HR, and Management all want to know, "How much of this cell therapy are you really going to sell, and when?" This interactive discussion will address:

- Basics of forecasting for both allogeneic and autologous cell therapies, in order to support decision making for clinical development, investor presentations, and manufacturing capacity;
- Determining realistic patient numbers, the impact of reimbursement on sales, the impact of projected sales on GMP manufacturing capacity;
- Staffing needs to support a given forecast.

**6:20 Close of Day**

**THURSDAY, FEBRUARY 4**

## ENABLING TECHNOLOGIES FOR REGENERATIVE MEDICINE

**8:25 Chairperson's Remarks**

*Marc Unger, Ph.D., CSO, Fluidigm*

**8:30 Human Embryonic Stem Cells (hESCs) for Tissue  
Regeneration: How to Get the Cells We Need**

*Harold S. Bernstein, M.D., Ph.D., Professor of Pediatrics, Eli and Edythe Broad Center of Regeneration Medicine & StemCell Research, University of California, San Francisco Cardiovascular Research Institute*

Cell therapies derived from hESCs have shown promise in animal models of human disease. However in some cases, such as in attempts to augment myocardial tissue, fully differentiated hESC-derived cells may be beyond the ability to fully incorporate into and improve the function of existing tissue. To address this, we have focused on identifying subpopulations of hESCs that preferentially differentiate into specific embryonic germ layers, developing chemical and miRNA-based enrichment strategies for directed differentiation of hESCs, and creating reporter hESC lines and non-integrating molecular beacons that facilitate the selection of precursors committed to specific lineages.

**9:00 Engineering the Morphogenesis of Pluripotent Stem Cells**

*Todd McDevitt, Ph.D., Assistant Professor, Wallace H. Coulter Department of Biomedical Engineering, Georgia Institute of Technology and Emory*

**9:30 Improved Culture Conditions for the Growth and  
Recovery of Cryopreserved Human Pluripotent Stem Cells**

*Angie Rizzino, Ph.D., Professor, Eppley Institute for Research in Cancer and Allied Diseases, University of Nebraska Medical Center*

Poor recovery of cryopreserved hES cells and iPS cells is a significant impediment to progress with pluripotent stem cells. To address this problem, we have determined that Y-27632, a specific inhibitor of Rho kinase (ROCK) activity, significantly enhances recovery of hES cells from cryopreserved stocks when cultured with or without a growth inactivated feeder layer. Remarkably, hES cells that had formed relatively few colonies even seven days after thawing exhibited rapid growth upon addition of Y-27632. Additionally, we determined that Y-27632 significantly improves the recovery of cryopreserved human iPS cells and their growth upon subculture.

## 10:00 Presentation

Sponsored by  Fluidigm

### Programmable, Fully Automated Microfluidic Stem Cell Culture System

Marc A. Unger, Ph.D., CSO, Fluidigm Corporation

Cell reprogramming techniques require treating cells with multiple factors, either for conversion of differentiated cells into induced pluripotent stem (iPS) cells or for conversion of pluripotent cells into a desired type of differentiated cells. Fluidigm is developing a versatile, automated cell culture system which can culture cells, carry out multi-factor dosing experiments, and image the cells in both fluorescence and incident light modes in any desired time sequence. The results of in-chip cell culture and multi-factor dosing experiments will be described and applications discussed.

## 10:30 Poster Competition Refreshment Break & Raffles in the Exhibit Hall

### 11:30 Integrated Chemical Genomics Reveals Modifiers of Cell Fate in Pluripotent Stem Cells

April Pyle, Ph.D., Assistant Professor, Microbiology, Immunology & Molecular Genetics, Eli and Edythe Broad Center of Regenerative Medicine & Stem Cell Research; Jonsson Comprehensive Cancer Center, University of California, Los Angeles

While hESCs can be maintained *in vitro*, cells grown in continuous culture have been shown to develop cytogenetic and genetic aberrations associated with cancer *in vivo*. Additionally, hESCs exhibit poor survival as single cells following dissociation, which limits the ability to perform genetic manipulation and homogenous differentiation of hESCs. In order to identify pathways involved in regulating self-renewal and survival without instability, we have developed a cell-based high content screening (HCS) assay using small molecules. This method provided a comprehensive approach for studying hESC fate *in vitro* and identified a number of novel regulators of hESC growth.

### 12:00 PM Biocompatible Grafted Carbon Nanotubes as Scaffolds for Preferential hESC Differentiation

Jennifer Lu, Ph.D., Professor, School of Engineering, University of California, Merced

Presented is our research on using biocompatible grafted carbon nanotubes as scaffolds for preferential neuron cells differentiation from hESCs. It has been found that carbon nanotube-based scaffolds promote the growth factor adsorption, leading to more selective differentiation. It has been observed that surface properties such as hydrophilicity and charge can play important roles in directing hESC differentiation. Novel responsive scaffolds have been synthesized and the potential use of such dynamic scaffolds for cell growth, differentiation and proliferation will be discussed.

## 12:30 Luncheon Presentation

Sponsored by



### Multiplex Biomarker Assays for Translational Research

Meso Scale Discovery

Robert Umek, Ph.D., Director of Research, Meso Scale Discovery

## 1:45 Ice Cream Refreshment Break in the Exhibit Hall

### PLENARY KEYNOTE SESSION

### 2:15 Plenary Keynote Introduction

### 2:25 Plenary Keynote Presentation (See Page 26 for Details)

### 3:05 Refreshment Break in the Exhibit Hall

### IPS CELLS: FROM SCREENING TO THERAPY

Induced pluripotent stem cells (iPS) cells, the most recent advancement in stem cell research, even further widen and generate applications for stem cell research. iPS cells exhibit great promise in drug discovery and screening as well as in regenerative medicine. iPS Cells: From Screening to Therapies not only explores current methods of generating, maintaining, and utilizing iPS cells but will address the shift in using them to contribute to Shaping the Future of Regenerative Medicine.

### 3:45 Chairperson's Remarks

Bruce Conklin, M.D., Senior Investigator, Gladstone Institute of Cardiovascular Disease, Professor of Medicine, Division of Medical Genetics, University of California, San Francisco

### IPS CELLS FOR DISEASE MODELS

### 3:50 Featured Speaker

### Potential Promise of iPS Cells for Understanding Disease Progression

Sheng Ding, Ph.D., Assistant Professor, Chemistry, Scripps Research Institute

### 4:20 iPS Cells for Cardiovascular Models and Diagnostics

Bruce Conklin, M.D., Senior Investigator, Gladstone Institute of Cardiovascular Disease, Professor of Medicine, Division of Medical Genetics, University of California, San Francisco

Our functional genomic experiments focus on GPCR signaling pathways in pluripo-

tent ES cell-derived cardiac myocytes. We use high-throughput gene inactivation methods, including siRNA and gene trapping in ES cells, and then analyze ES cell-derived cardiomyocytes. Our initial signaling studies focused on mouse ES cells, and ES cell-derived mice. We are using human iPS cells for similar signaling studies and to produce models of human cardiac disease including Long QT syndrome.

### 4:50 Using iPS Cells to Model Neurological Diseases

Clive N. Svendsen, Ph.D., Professor, Anatomy & Neurology, University of Wisconsin

### 5:20 Hoseok Song, Ph.D., Professor of Biology, University of California, San Diego

### 5:50 Close of Day

## FRIDAY, FEBRUARY 5

### IPS CELLS FOR REGENERATIVE HEALING

### 8:30 AM Chairperson's Opening Remarks

Timothy J. Kamp, M.D., Ph.D., Professor of Medicine & Physiology; Co-Director Stem Cell and Regenerative Medicine Center, University of Wisconsin

### 8:35 microRNA-Target Gene Networks as Fundamental Factors in the Next Generation Regenerative Strategies

Preethi H. Gunaratne, Ph.D., Assistant Professor, Department of Biology & Biochemistry, University of Houston

MicroRNAs are small non-coding RNAs that integrate multiple genes within and across biological pathways. LIN28/let-7; c-MYC-E2F/miR-17-92 and Oct4/Sox2/miR-302-cyclin D1 networks have been tightly linked to embryonic (ES) and more recently to iPS cells. We have also uncovered additional miRNAs regulated by Ronin, a non-canonical pluripotency factor that target genes regulating cytoskeletal remodeling and epigenetic silencing. Discussed is the potential role of these key miRNAs in the next generation regenerative strategies.

### 9:05 iPS Cells Offer New Alternative and Early Treatment in Genetic Diseases

Yuet Wai Kan, MB, BS, D.Sc., Professor, Department of Medicine, University of California, San Francisco

Two alternatives are currently available to parents if the prenatal diagnosis of a serious genetic disease is made: to terminate the pregnancy, or to continue it and take care of a seriously ill child. Generation of iPS cells from the amniotic fluid or CVS cells used for the diagnosis, correction of the mutation, and differentiation of the cells into specific tissues may in the future offer a new alternative. In addition, it will allow early treatment of the genetic disease, an important consideration in diseases where organ damage begins early in life.

### 9:35 Pluripotent Stem Cells Derived from Adult Human Testes

Martin Dym, Ph.D., Professor, Biochemistry & Molecular & Cellular Biology, Georgetown University Medical Center

Male germline stem cells obtained from adult human testes can be reprogrammed spontaneously to generate pluripotent stem cells. The production of these "non-canonical" iPS cells is spontaneous, and do not require the addition of exogenous genes, some of which may be cancer causing. Our results suggest that human spermatogonial stem cells have great potential for cell-based, autologous organ regeneration therapy for various diseases and it is thus possible that in the near future men could be cured of disease with a biopsy of their own testes.

### 10:05 Poster Presentation: Differentiation of Human Embryonic and Human Induced Pluripotent Stem Cells Along the Otic Lineage

Kinuko Masaki, Stanford University

### 10:20 Coffee Break

### IPS CELLS FOR REGENERATIVE HEALING CONT'D

### 11:00 Directed Differentiation of Human iPS Cells Generates Active Motor Neurons

William Lowry, Ph.D., Assistant Professor, Department of Molecular, Cell and Developmental Biology, University of California, Los Angeles

A study of gene expression profiles of mouse and human ESCs and iPSCs suggests that, while iPSCs are quite similar to their embryonic counterparts, a recurrent gene expression signature appears in iPSCs regardless of their origin or the method by which they were generated. Shown is how both hESCs and hiPSCs can differentiate to form fully functional differentiated progeny. We are now setting out to understand whether the differentiated progeny of hESCs and hiPSCs share commonalities or differences as was observed in their undifferentiated parent cells in an attempt to make predictions about whether these two types of pluripotent cells have similar potentials in regenerative medicine.



### 11:30 Functional Cardiomyocytes Derived from Human Induced Pluripotent Stem Cells

Timothy J. Kamp, M.D., Ph.D., Professor of Medicine & Physiology; Co-Director Stem Cell and Regenerative Medicine Center, University of Wisconsin

Human iPS cells hold great promise for cardiovascular research and therapeutic applications, but the ability of human iPS cells to form functional cardiomyocytes requires careful analysis and optimization. We provide electrophysiological, pharmacological, and biochemical evidence that iPS cells can differentiate into the three major types of functional cardiomyocytes which can be used in a variety of applications.

### 12:00 PM Lunch on Your Own

#### IPS CELLS FOR DRUG SCREENING


### 1:00 Chairperson's Remarks

#### 1:05 Featured Speaker

#### Stem Cells and Drug Discovery: The Beginning of a New Era?

Lee Rubin, Ph.D., Director, Translational Medicine, Harvard Stem Cell Institute

#### 1:35 CATALYST: The Industrialization of

Advanced iPSC Technology for Drug Discovery  Fate Therapeutics

Dan Shoemaker, Chief Technology Officer, Fate Therapeutics

CATALYST is designed to accelerate the innovation of induced pluripotent stem cell (iPSC) technology in collaboration with industry to support launching a fully-enabled platform in this new paradigm of drug discovery and development. CATALYST is exploring the creation of iPSC-derived, disease-specific model systems that improve the recapitulation of human physiology and more effectively predict clinical response. CATALYST is committed to developing an iPSC cell technology platform to accelerate candidate identification and lead validation for drug discovery and development for pharmaceutical members. This session will discuss:

- The critical elements of industrialization, including cell sourcing, reprogramming, differentiation and commercial supply
- Approaches for creating non-genetically modified iPS cells and mature phenotypes
- Quantitative methods to analyze cell states for high-quality differentiation and disease modeling
- Uses of iPSC technology in drug discovery

#### 1:50 Sponsored Presentation

Sponsored by



#### Targeting Muscular Dystrophy: How do we Mimic the In Vivo System?

Lorena Griparic, Ph.D., Research Scientist, DV Biologics

Muscular dystrophy (MD) is a well characterized neuromuscular disorder. Here we show that using cells isolated from different tissues of MD patients and their pedigree is an effective tool for understanding how to treat the disease. Our MD pedigree system is the first commercially available tool allowing the study of this disease and the production of iPS cells.

#### 2:05 iPSC-enabled Drug Discovery: A Paradigm Shift to Increase POS in the Clinic

Sponsored by



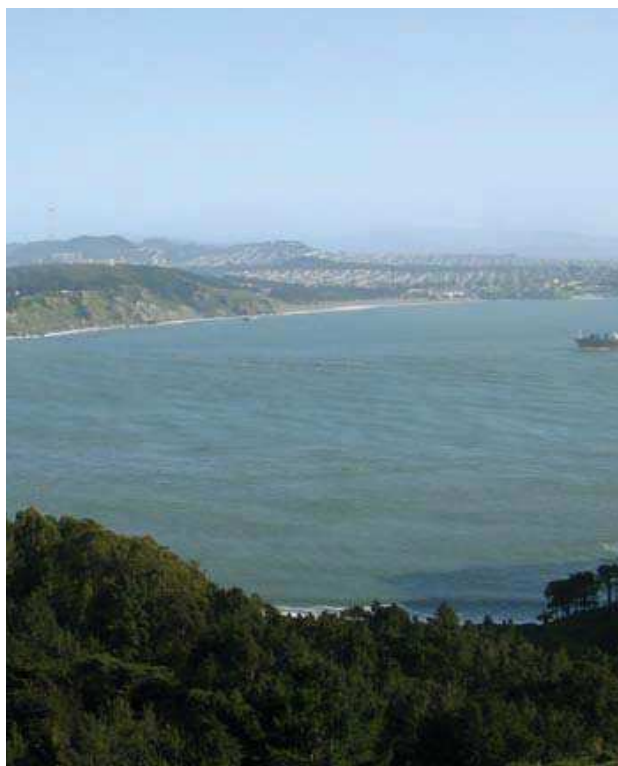
Berta Strulovici, Chief Technology Officer, iPierian

Until recently, human disease specific pluripotent stem cells could be made only by tedious genetic modification of existing hES cells or by generating such cells from embryos with diagnosed monogenic diseases. Recent advances using induced pluripotent stem cells (iPSCs) have enabled the production of unlimited numbers of cells with a very specific genetic background that can be used as models for drug discovery. Coupled with the ability of these cells to be differentiated to virtually "any type of cell in the body", the iPSC technology has the ability to revolutionize the way drug discovery is done today. In my presentation, I will describe the use of human iPSC-based assays for drug discovery in our therapeutic areas of focus.

#### 2:20 iPS Cells Panel of Experts

iPS cells have invigorated and united the stem cell research community and strides continue in efficient re-programming. This is evident through funding and companies investing their future through this revolutionary technology. Hear the experts in the iPS Cells field as they present their latest technology followed by an interactive panel discussion.

#### 3:05 Close of Conference



## HOTEL & TRAVEL INFORMATION

Conference Venue  
The Moscone North Convention Center  
747 Howard Street  
San Francisco, CA 94103  
[www.moscone.com](http://www.moscone.com)  
Host Hotel

Marriott San Francisco Hotel  
55 Fourth Street  
San Francisco, CA 94103  
Discounted Group Rate: \$199\* s/d  
\*Room Rate includes  
complimentary internet  
access in your guestroom  
Discounted Room Rate Cut Off Date:  
January 6, 2010  
(T) 415-896-1600  
(F) 415-486-8101

Minutes from the Moscone Convention Center, discover the beautiful San Francisco Marriott rising 39 stories high into the city skyline. Just south of Market Street, the hotel is steps away from the city's top attractions, including the Yerba Buena Gardens, world-class shopping on Union Square, and AT&T Park, home of the San Francisco Giants. Enjoy magnificent views of downtown San Francisco.

Please visit the conference website to make your hotel reservation on-line. You may also call the hotel directly to make your reservation by asking for the Cambridge Healthtech and/or Molecular Medicine Tri-Conference group rate. Reservations made after the cut-off date or after the group room block has been filled (whichever comes first) will be accepted on a space and-rate availability basis. Rooms are limited, so please book early.

For additional Travel Information, visit the hotel and travel page of the conference website [tri-conference.com](http://tri-conference.com).

**Reserve your hotel and save \$75 off your conference registration.\***

\*You must book your reservation under the Molecular Medicine Tri-Conference room block for a minimum of three nights at the Marriott San Francisco Hotel.

# Cambridge Healthtech Institute's Second Annual RNA Interference: From Tools to Therapies

**WEDNESDAY, FEBRUARY 3**

**7:00 AM Registration and Morning Coffee**

**8:00 Plenary Keynote Session** (See Page 26 for Details)

**9:40 Grand Opening Refreshment Break in the Exhibit Hall**

## THE POWER AND POTENTIAL OF WHOLE GENOME RNAi SCREENS

**11:00 Chairperson's Remarks**

*Christophe J. Echeverri, Ph.D., CEO and CSO, Cenix BioScience GmbH*

**11:10 Genome-wide RNAi Screen in *Drosophila* Cells Identifies G Protein-coupled Receptor Kinase 2 as an Evolutionarily Conserved Regulator of the NF- $\kappa$ B Signaling**

*Mika Rämetsä, M.D., Ph.D., Professor of Experimental Pediatric Immunology and Infectious Diseases, University of Tampere, Finland*

We have carried out an RNA interference-based genome-wide *in vitro* reporter assay screen in *Drosophila* for components of NF- $\kappa$ B pathways. We analyzed 16,025 dsRNA-treatments and identified ten novel NF- $\kappa$ B regulators. Of these, Gprk2 was shown to be evolutionarily conserved regulator of NF- $\kappa$ B signalling. siRNA-silencing of human ortholog GRK5 in HeLa cells impaired NF- $\kappa$ B reporter activity. Morpholino-silencing of zebrafish GRK5 homolog in fish embryos caused impaired IL-1 $\beta$  and TNF- $\alpha$  expression after *E. coli* infection. Gprk2/GRK5 was identified as an evolutionarily conserved modulator of NF- $\kappa$ B signaling.

**11:40 Alexander Bishop, Ph.D., Associate Professor, UT San Antonio**

**12:10 PM Generation And Integration of HT-RNAi Screening Data**

*Pedro Aza-Blanc, Ph.D., Director, Functional Genomics Resources, The Burnham Institute for Medical Research*

**12:40 Luncheon Presentation I**

**Using siRNA to Investigate Non-Coding RNA (ncRNA) Function in Control of Mitosis and Apoptosis in Cells**

*Susan Magdalena, Ph.D., Senior Manager, Scientist, RNAi Technologies Research & Development, Applied Biosystems*

Long non-coding RNAs (ncRNA) are critical to biology and disease. Life Technologies has now developed a suite of integrated tools and workflows to discover, validate, and knock-down ncRNA which will accelerate understanding of the function of ncRNA in the cell. We will describe the special requirements for using siRNAs to knock down ncRNA and will highlight the application of siRNAs to investigate ncRNA function in regulating mitosis and apoptosis in normal and cancer cells.

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**1:10 Luncheon Presentation II** (Sponsorship Opportunity Available)

**1:45 Dessert in the Exhibit Hall**

## EXPLORING HIGH CONTENT RNAi SCREENS

**2:15 Chairperson's Remarks**

*Paul Kassner, Ph.D., Scientific Director, Lead Discovery, Amgen, Inc.*

**2:20 Use of Fluorescence Microscopy to Track Protein Localization in siRNA Screening**

*Paul Kassner, Ph.D., Scientific Director, Lead Discovery, Amgen, Inc.*

**2:50 Comparative Analysis of RNAi Screening Performance Across Multiple Kinase-Focused Libraries: How Good is a Good Kinase Targeting Sequence?**

*Hakim Djaballah, Ph.D., Director, HTS Core Facility, Memorial Sloan Kettering Cancer Center*

We have assembled and obtained several kinase focused libraries for use in the comparative analysis of RNAi knockdown performance. We have performed a systematic RNAi screening of the Kinome represented in several siRNA and shRNA libraries for gene inactivation that modulate apoptotic events in an isogenic pair of cell lines, namely HeLa/B5 and HeLa/N10. HeLa B5 is stably transfected and over expresses Bcl-XL, a member of the anti-apoptotic Bcl-2 family, whereas the HeLa N10 contains an empty expression vector as a control. We have employed a high content assay measuring real time induction of apoptosis in live cells together with an end point measure of nuclear count and morphological changes post fixation and staining. We will present and discuss our findings.

**3:20 Speaker to be Announced**

**3:50 Sponsored Presentation** (Sponsorship Opportunity Available)

**4:20 Reception in the Exhibit Hall** (Sponsorship Available)

**5:20 BREAK-OUT DISCUSSIONS in the Exhibit Hall**

**Design of HT-RNAi Screens for Target Identification and Validation**

*Moderators: Paul Kassner, Ph.D., Scientific Director, Lead Discovery, Amgen Inc.*

*Christopher Miller, Ph.D., Group Director, Applied Genomics, Bristol-Myers Squibb Co.*

**Topics for discussion:**

- Choice of screening format, libraries and reagents
- Design of positive and negative controls
- Addressing the issue of off-target effects
- Statistical approaches for prioritizing hits

**Perspectives on siRNA Delivery Systems**

*Moderators: Ian MacLachlan, Ph.D., Chief Scientific Officer, Tekmira Pharmaceuticals Inc.*

*Antonin de Fougerolles, Ph.D., Vice President, Research, Alnylam Pharmaceuticals Inc.*

**Topics for discussion:**

- Key attributes necessary for delivery
- Pharmaceutical aspects of siRNA formulations
- Formulation-specific safety questions
- Immune activation and RNAi delivery

**Progress in Developing RNAi Therapeutics**

*Moderator: Bob Brown, Ph.D., Senior Vice President, Research, Dicerna Pharmaceuticals Corp.*

**Topics for discussion:**

- Transitioning from the lab to the clinic
- Challenges in the clinic
- RNAi therapies for acute versus chronic conditions
- Lessons learnt from gene therapy and antisense

**6:20 Close of Day**

## THURSDAY, FEBRUARY 4

### SCREENING AND VALIDATING DRUG TARGETS USING RNAi SCREENS

#### 8:25 AM Chairperson's Remarks

Christopher Miller, Ph.D., Group Director, Applied Genomics, Bristol-Myers Squibb Co.

#### 8:30 Cancer Target Identification and Validation by siRNA

##### Library Screening

Xiaoyu Lin, Ph.D., Associate Research Investigator, siRNA Therapeutics, Abbott Laboratories

We have been performing large-scale siRNA library screens to identify novel cancer targets. One of the critical aspects of screen data analysis is to discard false positive hits due to siRNA off-target effect. We will discuss several different approaches to confirm on-target effect of siRNA library hits. Using several targets identified in the library screen as examples, we will update on the progress of how RNAi-based technologies have helped target discovery and validation in the oncology area.

#### 9:00 Hitting Cancer Where it Hurts Most: Large Scale RNAi Screens for Cancer Cell Vulnerability

Roderick L. Beijersbergen, Ph.D., Group Leader, Division of Molecular Carcinogenesis, The Netherlands Cancer Institute

Large scale RNAi screens for cancer cell vulnerability RNA interference based technologies allow for the interrogation of the role and phenotype of all individual genes in the human genome. Using these techniques we aim to identify those genes that upon functional inactivation have a causal effect on tumor cell behavior and survival representing novel drug targets.

#### 9:30 Leveraging RNAi and Chemogenomic Screens for Target Identification and Validation

Christopher Miller, Ph.D., Group Director, Applied Genomics, Bristol-Myers Squibb Co.

Libraries of RNAi reagents are being widely used for screening cellular assays for target identification. We are using RNAi libraries in combination with libraries of small molecule tool compounds. Using both types of libraries adds confidence to hits identified from these screens and provides genetic and chemical tools for hit follow up. Examples will be presented to illustrate this approach in target identification and validation.

#### 10:00 Sponsored Presentation (Sponsorship Opportunity Available)

#### 10:30 Poster Competition Refreshment Break & Raffles in the Exhibit Hall

#### 11:30 Panel: Do's and Don'ts in RNAi Screening

##### Panelists:

Christophe J. Echeverri, Ph.D., CEO and CSO, Cenix BioScience GmbH

Christopher Miller, Ph.D., Group Director, Applied Genomics, Bristol-Myers Squibb Co.

Hakim Djaballah, Ph.D., Director, HTS Core Facility, Memorial Sloan Kettering Cancer Center

Paul Kassner, Ph.D., Scientific Director, Lead Discovery, Amgen, Inc.

#### 12:30 PM Luncheon Presentation RNAi and KinaseSwitch Technology Platforms

Sponsored by

Christine L. Olsson, Ph.D., Taconic

Novel *in vivo* technology platforms have recently been developed that will enable investigators to gain greater insights into drug and target-related disease mechanisms. TaconicArtemis RNAi and KinaseSwitch mouse models are the newest commercially available additions to these technologies. Inducible/reversible RNAi technology enables gene knockdown in all tissues of the body and can be induced and reversed, providing an optimal surrogate for therapeutic drug action. More recently, KinaseSwitch technology, an invaluable tool during key stages of drug development, allows investigators to identify biological roles of a specific kinase and possible side effects that result from its inhibition.

#### 1:45 Ice Cream Refreshment Break in the Exhibit Hall

## PLENARY KEYNOTE SESSION

#### 2:15 Plenary Keynote Introduction

#### 2:25 Plenary Keynote Presentation (See Page 26 for Details)

#### 3:05 Refreshment Break in the Exhibit Hall

### CLINICAL CHALLENGES WITH RNAi THERAPEUTICS

#### 3:45 Chairperson's Remarks

Cristina Rondinone, Ph.D., Director Research, Metabolic Diseases, Hoffmann La Roche Inc.

#### 3:50 LNA Antimirs – Pioneering microRNA Therapeutics

Henrik Orum, M.Sc., Ph.D., VP and CSO, Santaris Pharma

Short, single stranded LNA oligonucleotides delivered systemically as naked molecules are able to potently and safely inhibit therapeutically attractive miRNAs in a range of tissues in experimental animals. The presentation will provide an update on the unique features of LNA oligonucleotides in miRNA therapeutics with particular emphasis on the pre-clinical and clinical development of SPC3649, an LNA AntimiR targeting miRNA-122.

#### 4:20 Pre-clinical and Clinical Development of Atu027 (siRNA-lipoplex/AtuPLEX) for Oncology

Ansgar Santel, Ph.D., Senior Scientist, Silence Therapeutics plc

Atu027 refers to a liposomally formulated siRNA targeting PKN3 expression in the vascular endothelium. Pre-clinical studies in rodents and non-human primates demonstrated that intravenous administration is well tolerated and gives rise to RNAi-mediated suppression of PKN3 gene expression. Various proof-of-concept experiments on mouse tumor xenografts suggest profound inhibition of tumor progression and particularly of metastasis, which laid the foundation for therapeutic application in oncology. Atu027 is currently tested in a Phase-I clinical trial on subjects with advanced solid cancers. Pre-clinical data emphasizing pharmacological activity in mouse models and an update on the current Phase-I study will be discussed.

#### 4:50 Talk Title to be Announced

#### 5:20 Talk Title to be Announced

Bob D. Brown, Ph.D., Senior Vice President, Research, Dicerna Pharmaceuticals Corp.

#### 5:50 Close of Day

## FRIDAY, FEBRUARY 5

### NOVEL FORMULATIONS FOR RNAi DELIVERY

#### 8:30 AM Chairperson's Opening Remarks

Bob D. Brown, Ph.D., Senior Vice President, Research, Dicerna Pharmaceuticals, Corp.

#### 8:35 Therapeutic siRNA Delivery: Tackling the 800 Pound Gorilla

TaconicArtemis

Steven F. Dowdy, Ph.D., Investigator, Howard Hughes Medical Institute; Professor, Department of Cellular & Molecular Medicine, University of California, San Diego School of Medicine

To date, siRNA delivery remains the rate-limiting step for RNAi therapeutics development. We developed a Peptide Transduction DomainsRNA Binding Domain (PTD-DRBD) fusion protein siRNA delivery approach. PTD-DRBD delivered siRNAs induced RNAi responses in the entire population of all cell types assayed (primary and tumorigenic) in a non-cytotoxic fashion. PTD-DRBD combinatorial *in vivo* delivery of EGFR and Akt2 siRNAs induced a synthetic lethal response that significantly increased survival of intracerebral glioblastoma pre-clinical models. These observations demonstrate the ability of PTD-DRBD to efficiently deliver siRNAs *in vivo*.

### 9:05 Induction of Therapeutic Gene Silencing in Leukocyte-Implicated Diseases by Targeted and Stabilized Nanoparticles

Dan Peer, Ph.D., Head, Laboratory of Nanomedicine, Department of Cell Research and Immunology and the Center for Nanoscience and Nanotechnology, Tel Aviv University

Leukocytes are among the most difficult cells to transduce with RNAi. We developed a strategy that can target different subsets of leukocytes and selectively silence genes *in vivo* using targeted, stabilized nanoparticles (tsNPs). These carriers do not induce lymphocyte activation, interferon responses or release liver enzymes and are fully degradable. Three pre-clinical examples inflammatory bowel disease (IBD), blood cancer and viral infection will be discussed. We will show that tsNPs can be used for *in vivo* validation of new drug targets, for prevention of viral infection and for inducing therapeutic gene silencing in a preclinical setting.

### 9:35 Characterization of Immune Responses to tkRNAi Therapeutics

Johannes Fruehauf, M.D., Ph.D., VP, Research, Cequent Pharmaceuticals, Inc.

Transkingdom RNA interference (tkRNAi) describes a novel method for delivery of therapeutic RNA interference into gastrointestinal tissues using engineered bacteria which produce and deliver mediators of RNAi. Clinical trials are about to begin for the prevention of colon Polyposis, and for the treatment of Inflammatory Bowel Disease (IBD). Here we demonstrate recent results from large screening efforts characterizing the effects of tkRNAi on cytokine profiles and innate immunity.

### 10:05 Sponsored Presentation (Sponsorship Opportunity Available)

### 10:20 Coffee Break

### 11:00 Panel: Do We Understand the Challenges We Face With RNAi Therapeutics?

Panelists:

Bob D. Brown, Ph.D., Senior Vice President, Research, Dicerna Pharmaceuticals Corp.

Ian MacLachlan, Ph.D., CSO, Tekmira Pharmaceuticals

John Rossi, Ph.D., Professor and Chair, Molecular Biology, Beckman Research Institute of the City of Hope

Steven Highlander, Ph.D., Partner, Intellectual Property, Fulbright & Jaworski, L.L.P.

### 12:00 PM Luncheon Presentation (Sponsorship Opportunity

Available) or Lunch on Your Own

## NOVEL APPROACHES FOR TARGETED DELIVERY

### 1:00 Chairperson's Remarks

John Rossi, Ph.D., Professor and Chair, Molecular Biology, Beckman Research Institute of the City of Hope

### 1:05 *In vivo* delivery of Dicer substrate RNAs for treatment of HIV infection

John Rossi, Ph.D., Professor and Chair, Molecular Biology, Beckman Research Institute of the City of Hope

The application of RNAi for treatment of HIV infection has many advantages over conventional drugs. The inhibitors can be rapidly changed according to the viruses ability to mutate. This presentation will discuss the use of aptamers and dendrimers to deliver Dicer substrate RNAs *in vivo*. The results obtained demonstrate that Dicer substrate siRNAs can be delivered in a cell type specific manner with an aptamer, and can be generally delivered with dendrimers to effectively inhibit HIV replication in a humanized mouse model.

### 1:35 Targeted RNA-based Cancer Therapies

Paloma H. Giangrande, Ph.D., Assistant Professor, Department of Internal Medicine, University of Iowa

A major hurdle for the clinical translation of siRNAs into effective therapies is delivery. We describe an RNA aptamer-based approach for the targeted delivery of siRNAs to prostate cancer (PC) cells. The aptamer-siRNA reagent (chimera) is effective when administered systemically and is suitable for efficient chemical synthesis. When administered systemically to mice bearing PSMA-positive tumors, the RNA chimera triggered tumor regression without affecting normal tissues. This work is the first description of *in vivo* efficacy following systemic administration of an aptamer-siRNA chimera and thus represents a milestone for this platform technology.

### 2:05 Development of Novel Therapeutic RNAi Compounds and Effective *in vivo* Delivery Approaches

Joanne Kamens, Ph.D., Senior Director of Research Collaboration Management, RXi Pharmaceuticals

RNA interference (RNAi) offers a novel approach to the drug development process, because RNAi compounds can potentially be designed to target any one of the genes in the human genome. Other potential advantages of RNAi therapeutics include, rapid development of lead compounds, high selectivity for the target gene, high potency (low dose) and low toxicity due to natural mechanism of action. We will introduce unique single-oligo (rxRNA<sup>solo</sup>) and short-duplex (rxRNA<sup>nano</sup>) RNAi compounds, as well as novel *in vivo* delivery approaches, including self-delivering rxRNA molecules (sd-rxRNA) for local and systemic delivery, and targeted delivery to phagocytic immune cells using.

### 2:35 Development of RNA Interference Therapeutics

Antonin De Fougerolles, VP, Research, Alnylam Pharmaceuticals

Novel therapies based on short interfering RNA (siRNA) duplexes have tremendous potential to treat diseases by silencing the expression of otherwise non-druggable proteins. Development of therapeutics using siRNA has advanced rapidly, with multiple different clinical trials ongoing and several more poised to enter the clinic in the coming years. Although challenges remain, delivery represents the main hurdle for faster and broader development of siRNA therapeutics. A summary of the advances made around siRNA delivery will be presented.

### 3:05 Close of Conference



# Cambridge Healthtech Institute's Inaugural Cancer Biologics

WEDNESDAY, FEBRUARY 3

7:00 AM Registration and Morning Coffee

8:00 Plenary Keynote Session (See Page 26 for Details)

9:40 Grand Opening Refreshment Break in the Exhibit Hall

## DELIVERY OF CANCER BIOLOGICS PENETRATION AND DISTRIBUTION

11:00 Introduction and Welcome

Gregory P. Adams, Ph.D., Co-Leader, Molecular and Translational Medicine Program, Fox Chase Cancer Center

11:10 Tumor Penetration of Therapeutic Antibodies -The Impact of Size and Exposure Time on Distribution

David Blakey, Ph.D., Chief Scientist, Cancer and Infection Research Area, AstraZeneca

The ability of intact antibodies and fragments to access tumor cells distant from the tumor blood supply is an important therapeutic consideration for antibody based oncology drugs. Pre-clinical and clinical data will be reviewed regarding the impact of size and exposure time on antibody distribution within tumors.

11:40 Anti-tumor Efficacy Maximization through Blocking Multiple Targets of Angiogenesis

Dana Hu-Lowe, Ph.D., Group Leader, Associate Research Fellow, Cancer Biology, Pfizer, Inc., PGDR-La Jolla

Vascular normalization and adaptively potentially contribute to resistance to anti-VEGF/VEGFR therapies in the clinic. Other targets, including the Activin receptor Like Kinase 1 (ALK-1), also play a role in promoting tumor angiogenesis. A fully human mAb against ALK-1 was generated. The differential and complimentary outcome of anti-ALK-1 and anti-VEGF will be discussed.

12:10 PM Optimizing Targeting of Anti-tumor Antibodies

Gregory P. Adams, Ph.D., Co-Leader, Molecular and Translational Medicine Program, Fox Chase Cancer Center

We have found that anti-HER2 scFv molecules penetrate and localize in a solid tumors less efficiently with increasing affinity. We will describe studies performed with anti-HER2 human IgG molecules that demonstrate that affinity also impacts the targeting of intact antibodies. Studies examining the roles of affinity on *in vitro* ADCC and internalization into tumor cells will also be discussed.

12:40 Luncheon Presentations (Sponsorship Opportunity Available) or Lunch on Your Own

1:45 Dessert in the Exhibit Hall

## SELECTIVE TARGETING OF TUMORS

2:15 Chairperson's Remarks

Matthew K. Robinson, Ph.D., Assistant Professor, Molecular and Translational Medicine Program, Fox Chase Cancer Center

2:20 Bispecific Antibodies: An Approach to Enhance Targeting Selectivity and Efficacy

Matthew K. Robinson, Ph.D., Assistant Professor, Molecular and Translational Medicine Program, Fox Chase Cancer Center

Work will be presented on our efforts to develop and optimize the targeting selectivity of bispecific antibodies that co-target two distinct tumor associated antigens. We hypothesize that the targeting selectiv-

ity afforded by these molecules can potentially be leveraged for the development of new immunodrug conjugates.

2:50 Selective Penetration and Targeting of Tumors

Tapas K. Das Gupta, M.D., Ph.D., D.Sc., Professor and Head, Surgical Oncology, University of Illinois Chicago; Co-founder, CDG Therapeutics, Inc.

CDG Therapeutics has developed a cell penetrating peptide (28aa) from azurin, a redox protein secreted by *Pseudomonas aeruginosa*. p28 preferentially enters cancer cells, localizes in the nucleus and stabilizes p53 inducing cell cycle arrest and apoptosis in a series of solid tumors. p28 is stable, nontoxic and currently in a Phase I clinical trial.

3:20 A Systems Biology Approach to Engineering Therapeutic Antibodies: Development of an ErbB2/ErbB3 Bispecific Antibody

Alexandra Huhlov, Ph.D., Principal Scientist, Merrimack Pharmaceuticals

Using quantitative biological datasets of cell signaling we have generated computational models of the ErbB signaling network and identified ErbB3 as a promising target. The application of these models to guide the design of MM-111, a bispecific antibody-based therapeutic targeting the ErbB2/ErbB3 heterodimer, and its antitumor activity will be discussed.

3:50 Large Volume Subcutaneous Delivery: Challenges and Opportunities

Robin Hwang, Ph.D., Executive Director, Halozyme

There are many monoclonal antibodies (mAbs) in development for cancer therapeutics. Generally, mAbs require a higher dosage than the typical protein therapeutics. It has been shown clinically that the "standard" subcutaneous injection can go up to 1.5 mL, beyond which skin distortion and pain can occur. As a result, most biotech companies spend much effort in concentrating mAbs to ~100 mg/mL and then trying to stabilize these formulations to avoid aggregates and particulates. Halozyme's Enhance™ Technology permits the large volume subcutaneous (SC) dosing, with corresponding lower protein concentration which was not previously feasible. Bypassing high-concentration formulation challenges has the potential to accelerate the timeline to bring a product to the clinic, enables an IV-SC switch, in some cases improves bioavailability, and improve patient convenience and compliance. In this talk, large volume SC delivery and device options will be presented.

4:20 Reception in the Exhibit Hall (Sponsorship Available)

5:20 BREAK-OUT DISCUSSIONS in the Exhibit Hall

6:20 Close of Day

THURSDAY, FEBRUARY 4

## ENGINEERING FOR DELIVERY

8:25 AM Chairperson's Remarks

Tugrul Kararli, Ph.D., President & Founder, Pharmacricle

8:30 Tumor Targeting Theory-Kinetic & Diffusive Processes that Determine Antibody Macro & Microdistribution

K. Dane Wittrup, Ph.D., C.P. Dubbs Professor, Chemical Engineering & Biological Engineering, Associate Director, Koch Institute for Integrative Cancer Research, Massachusetts Institute of Technology

A diverse array of tumor targeting agents ranging in size from peptides to nanoparticles is currently under development for applications in cancer imaging and therapy. However, it remains largely unclear how size differences among these molecules influence their targeting properties. Here

we develop a simple, mechanistic model that can be used to understand and predict the complex interplay between molecular size, affinity, and tumor uptake.

#### 9:00 Nanoparticle Agents for Tumor Targeting and Penetration

Shuming Nie, Ph.D., Wallace H. Coulter Distinguished Faculty Chair in Biomedical Engineering, Director of Emory-Georgia Tech Cancer Nanotechnology Center, Professor of BME, Chemistry, Materials Science and Engineering, and Hematology and Oncology, Emory University and Georgia Institute of Technology

Nanoparticles have functional and structural properties not available from discrete molecules or bulk materials. When conjugated with monoclonal antibodies, peptides or small molecules, nanoparticles can be used to target malignant tumors with high specificity and affinity. We developed a new class of biocompatible and nontoxic nanoparticles for *in vivo* tumor targeting and detection based on self-assembled nanostructures and pegylated colloidal gold.

#### 9:30 Delivery of Antibodies – Market Analysis & Overview

Tugrul Kararli, Ph.D., President & Founder, Pharmacircle

#### 10:00 Sponsored Presentation (Sponsorship Opportunity Available)

#### 10:30 Poster Competition Refreshment Break & Raffles in the Exhibit Hall

### DELIVERY OF ANTIBODIES

#### 11:30 Engineered Antibodies for Molecular Imaging of Cancer

Anna M. Wu, Ph.D., Professor, David Geffen School of Medicine at UCLA

Cancer-targeting antibodies have been optimized for *in vivo* imaging by conversion into fragments such as diabodies, minibodies, and scFv-Fc. Recombinant fragments recognizing a variety of cell-surface markers have been labeled with positron-emitting radionuclides (I-124, Cu-64, F-18) for positron-emission tomography (PET) detection of tumors in preclinical models. ImmunoPET represents a broad platform for conducting “immunohistochemistry *in vivo*” to address biological questions in living organisms, including target expression, target coverage, and response to therapy.

#### 12:00 Advanced Polymer Conjugate Technology for Optimization of Cancer Therapeutics

Christine Loehrlein, Ph.D., A.D., New Products and Technology Strategy Research, Nektar Therapeutics

Conjugation of a therapeutic agent to polyethylene glycol and other polymers is a general strategy that can be used to optimize pharmacological parameters of that drug, with the ability to affect both its efficacy and side effect profile. Nektar's Advanced Polymer Conjugate Technology platform can be used to enable a wide range of molecules, including proteins, peptides, small molecule oral and parenteral drugs, and antibody fragments. Nektar is currently using this approach to develop advanced oncolytics with sustained exposure to tumor cells, and exploring opportunities to extend this technology to other cancer therapeutics.

#### 12:30 Luncheon Presentation (Sponsorship Opportunity Available) or Lunch on Your Own

#### 1:45 Ice Cream Refreshment Break in the Exhibit Hall

### PLENARY KEYNOTE SESSION

#### 2:15 Plenary Keynote Introduction

#### 2:25 Plenary Keynote Presentation (See Page 26 for Details)

#### 3:05 Refreshment Break in the Exhibit Hall

### NOVEL MODES OF ACTION FOR CANCER BIOTHERAPEUTICS BI- AND TRI-SPECIFIC ANTIBODIES

#### 3:45 Chairperson's Remarks

Stefan Dübel, Ph.D., Director, Biotechnology, Technische Universität Braunschweig, Germany

#### 3:50 Mode of BiTE Antibody Action in Cancer Therapy

Patrick Baeuerle, Ph.D., CSO and Senior Vice President, Research & Development, Micromet AG

BiTE (bispecific T cell engager) antibody blinatumumab targets CD19 on B cell malignancies and has provided clinical proof of concept in phases 1 and 2. We will discuss the mode of BiTE antibody action in inducing highly efficient cancer cells lysis, and provide background on how BiTE antibodies are produced, are administered in the clinic, and have been pre-clinically developed.

#### 4:20 Catumaxomab (Removab), the First EC-approved Trifunctional Bispecific Antibody: The Road from Pre-clinical Development to Approval and Beyond

Diane Seimet, Ph.D., M.D.R.A., CSO and Executive Vice President, Fresenius Biotech GmbH

Catumaxomab, a targeted therapy for intraperitoneal treatment of malignant ascites, targets the epithelial cell-adhesion molecule (EpCAM) and CD3 evoking T-cell cytotoxicity on EpCAM-expressing tumor cells. The Fc-region of catumaxomab provides a third functional binding site, which binds and activates Fcγ-receptor-positive accessory cells. The development rationale, pre-clinical and clinical data, approval process and preparations for further clinical development will be presented.

#### 4:50 Bispecific EGFR-IGF1R Program

Eric Furfine, Ph.D., Senior Vice President, Research and Pre-clinical Development, Adnexus Therapeutics, a Bristol-Myers Squibb R&D Company

Adnectins offer several potential advantages compared to traditional targeted biologics, including speed of discovery, efficient manufacturing, and the ability to create multi-functional targeted products. We are currently advancing products combining two Adnectins to enable modulation of two distinct targets. We will present methods to engineer and optimize multi-specific Adnectins, and pre-clinical data on a bispecific Adnectin to EGFR and IGF1R.

#### 5:20 Multi-specific Antibody by Design

Changshou Gao, Ph.D., Principal Scientist, Antibody Discovery and Protein Engineering, MedImmune LLC

We'll discuss efforts to engineer and optimize multispecific antibody formats to address the challenges pertaining to bispecific and multispecific molecules, and provide data of our multispecific constructs with excellent expression level, great biophysical stability, good *in vitro* and *in vivo* activities, and potential manufacturing feasibility. Our multispecific constructs retain similar *in vivo* half-life and effector functions to their parental antibodies.

#### 5:50 Close of Day



FRIDAY, FEBRUARY 5

## ADVANCES IN ANTIBODY-DRUG CONJUGATES

### 8:30 AM Chairperson's Opening Remarks

Hans-Peter Gerber, Ph.D., Senior Director, Tumor Therapies, Wyeth Oncology Discovery

### 8:35 A Novel Minor Groove Binding Alkylating Agent for Antibody Targeted Chemotherapy of CD70 Expressing Cancers

Nils Lonberg, Ph.D., Senior Vice President and Scientific Director, Medarex, Inc.

An antibody drug conjugate comprising a CD70 targeting monoclonal antibody and a novel alkylating agent is now in Phase I clinical development for kidney cancer and lymphoma. The mechanism of action of this novel therapeutic, activated through a multistep mechanism including esterase mediated removal of a blocking group and protease mediated release of the cytotoxic drug, will be discussed.

### 9:05 Pre-clinical and Clinical Development of Calicheamicin Derivatives Conjugated to Monoclonal Antibodies

Hans-Peter Gerber, Ph.D., Senior Director, Tumor Therapies, Wyeth Oncology Discovery

Gemtuzumab ozogamicin (Mylotarg), a semi-synthetic derivative of calicheamicin linked to a humanized anti-CD33 monoclonal antibody, is approved for the treatment of AML. CMC-544 (inotuzumab ozogamicin), an anti-CD22 immuno-conjugate of calicheamicin, is currently being evaluated in B-cell non-Hodgkin's lymphoma (B-NHL) patients. I will describe the mechanism of action and the pharmacology of calicheamicin conjugates and provide an overview of clinical trials.

### 9:35 Antibody-Maytansinoid Conjugates: Demonstrating Benefit in the Treatment of Solid and Liquid Tumors

John M. Lambert, Ph.D., Executive Vice President and CSO, ImmunoGen, Inc.

Several new highly potent cell-killing agents such as derivatives of the anti-mitotic microtubule agent, maytansine, are currently being utilized in ADCs to achieve effective, well tolerated anticancer drugs. Several ADCs show encouraging efficacy in clinical trials, including T-DM1, currently being developed by Genentech using ImmunoGen's maytansinoid technology. New payloads for ADCs are realizing the promise of antibody-mediated delivery in cancer.

### 10:05 Broad Application of Scaffold Antibodies for Targeted Tumor Therapy

Gary Woodhutt, Ph.D., Vice President, Biology, CovX Research, A Pfizer organization

The progression of novel cancer therapeutics that have the potential of truly impacting disease requires the identification of targets that affect tumor growth combined with a modality capable of rapid exploitation of those targets either as monotherapy or in combination. We will describe how the use of bioconjugation to a proprietary scaffold antibody allows us to develop these therapeutics rapidly and effectively.

### 10:20 Coffee Break

## EMERGING NEW TECHNOLOGIES

### 11:00 Bi-specific, High Affinity T Cell Receptor Fusions as Anti-Cancer Therapeutics

Rebecca Ashfield, D.Phil., Senior Research Project Manager, Immunocore Ltd

Bi-specific TCRs, consisting of high affinity T cell receptors fused to an anti-CD3 scFv, are being developed for the treatment of several tumor types. The presentation will cover engineering of these reagents, demonstration of efficacy including animal models, and a discussion of the planned first-in-man clinical trial including toxicity testing, a challenge since the molecules are entirely human specific.

### 11:30 Chemosensitization of Cancer Cells by siRNA Using Targeted Nanogel Delivery

John F. McDonald, Professor and Director, Integrated Cancer Research Center, School of Biology, Georgia Institute of Technology

Targeted cancer therapy by RNA interference (RNAi) is a promising approach to silence genes *in vivo*. Delivery is a major hurdle for the development of RNAi therapeutics. We report on the successful use of hydrogel nanoparticles (nanogels) functionalized with peptides that specially target the EphA2 receptor to deliver small interfering RNAs (siRNAs) targeting EGFR to increase sensitivity to Taxane therapy.

### 12:00 PM Luncheon Presentation (Sponsorship Opportunity Available) or Lunch on Your Own

## EFFECTOR-ENHANCED BIOTHERAPEUTICS

### 1:00 Chairperson's Remarks

John F. McDonald, Professor and Director, Integrated Cancer Research Center, School of Biology, Georgia Institute of Technology

### 1:05 Human RNase Fusion Proteins for Tumor Therapy

Stefan Dübel, Ph.D., Director, Biotechnology, Technische Universität Braunschweig, Germany

RNases are non-toxic while in circulation but highly effective in cell killing after targeted internalization. An entirely human immunoenzyme against CD30+ lymphomas was constructed from a human scFv-Fc antibody fragment and a human RNase. It did not affect the human embryonal kidney used for its production but strongly inhibited proliferation of CD30+ lymphoma cells.

### 1:35 Glycoengineering for the Enhancement of Antibody Activity

Dennis Benjamin, Ph.D., Senior Director, Antibody Technologies, Seattle Genetics, Inc.

### 2:05 Antibody Fc Engineering to Enhance Cytotoxicity, Pharmacokinetics, and Pharmacodynamics

John R. Desjarlais, Ph.D., VP, Research, Xencor, Inc.

We have engineered the antibody Fc domain to enhance its affinity for Fc receptors, leading to a set of variants that confer high ADCC activity onto antibodies targeting a wide range of tumor targets. These variants also enhance anti-tumor activity in mouse models and cynomolgus monkeys. A phase I trial is underway to determine their effects in humans.

### 2:35 Arzerra™ (ofatumumab), a Novel Human Therapeutic CD20-Antibody: Mechanisms of Action and Efficacy in B-CLL

Frank Beurskens Ph.D., Senior Scientist, Strategic Research, Genmab, B.V.

We developed a unique human monoclonal CD20 antibody, ofatumumab, that targets a distinct epitope encompassing both the small and large extracellular loops of the CD20 molecule and displays an exceptional efficacy in inducing complement dependent cell lysis (CDC). Novel insights into the mechanisms of tumor cell killing by ofatumumab and its efficacy in clinical trials in B-CLL will be discussed.

### 3:05 Close of Conference

## ALUMNI DISCOUNT

Cambridge Healthtech Institute (CHI) appreciates your past participation at the Molecular Medicine Tri-Conference. Through loyalty like yours, CHI has been able to build this event into a must attend for senior level decision-makers. As a result of the great loyalty you have shown us, we are pleased to extend to you the exclusive opportunity to save an additional 20% off the registration rate. Just check off the box marked Alumni Discount on the registration form to receive the discount! Please note: Our records must indicate you were an attendee of the Tri-Conference event in the past in order to qualify.

Receive  
20% Off Your  
Registration!

CANCER & BIOLOGICS CHANNEL

# Cambridge Healthtech Institute's Inaugural Delivery of Biologics

## WEDNESDAY, FEBRUARY 3

**7:00 AM Registration and Morning Coffee**

**8:00 Plenary Keynote Session** (See Page 26 for Details)

**9:40 Grand Opening Refreshment Break in the Exhibit Hall**

### PENETRATION AND DISTRIBUTION

**11:00 Introduction and Welcome**

*Gregory P. Adams, Ph.D., Co-Leader, Molecular and Translational Medicine Program, Fox Chase Cancer Center*

**11:10 Tumor Penetration of Therapeutic Antibodies -The Impact of Size and Exposure Time on Distribution**

*David Blakey, Ph.D., Chief Scientist, Cancer and Infection Research Area, AstraZeneca*  
The ability of intact antibodies and fragments to access tumor cells distant from the tumor blood supply is an important therapeutic consideration for antibody based oncology drugs. Preclinical and clinical data will be reviewed regarding the impact of size and exposure time on antibody distribution within tumors.

**11:40 Anti-tumor Efficacy Maximization through Blocking Multiple Targets of Angiogenesis**

*Dana Hu-Lowe, Ph.D., Group Leader, Associate Research Fellow, Cancer Biology, Pfizer, Inc., PGRD-La Jolla*

Vascular normalization and adaptivity potentially contribute to resistance to anti-VEGF/VEGFR therapies in the clinic. Other targets, including the Activin receptor Like Kinase 1 (ALK-1), also play a role in promoting tumor angiogenesis. A fully human mAb against ALK-1 was generated. The differential and complimentary outcome of anti-ALK-1 and anti-VEGF will be discussed.

**12:10 PM Optimizing Targeting of Anti-tumor Antibodies**

*Gregory P. Adams, Ph.D., Co-Leader, Molecular and Translational Medicine Program, Fox Chase Cancer Center*

We have found that anti-HER2 scFv molecules penetrate and localize in a solid tumors less efficiently with increasing affinity. We will describe studies performed with anti-HER2 human IgGs molecules that demonstrate that affinity also impacts the targeting of intact antibodies. Studies examining the roles of affinity on *in vitro* ADCC and internalization into tumor cells will also be discussed.

**12:40 Luncheon Presentation** (Sponsorship Opportunity Available) or **Lunch on Your Own**

**1:45 Dessert in the Exhibit Hall**

### SELECTIVE TARGETING OF TUMORS

**2:15 Chairperson's Remarks**

*Matthew K. Robinson, Ph.D., Assistant Professor, Molecular and Translational Medicine Program, Fox Chase Cancer Center*

**2:20 Bispecific Antibodies: An Approach to Enhance Targeting Selectivity and Efficacy**

*Matthew K. Robinson, Ph.D., Assistant Professor, Molecular and Translational Medicine Program, Fox Chase Cancer Center*

Work will be presented on our efforts to develop and optimize the targeting selectivity of bispecific antibodies that co-target two distinct tumor associated antigens. We hypothesize that the targeting selectivity afforded by these molecules can potentially be leveraged for the development of new immunodrug conjugates.

**2:50 Selective Penetration and Targeting of Tumors**

*Tapas K. Das Gupta, M.D., Ph.D., D.Sc., Professor and Head, Surgical Oncology, University of Illinois Chicago; Co-founder, CDG Therapeutics, Inc.*

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**3:50 Sponsored Presentation** (Sponsorship Opportunity Available)

**4:20 Reception in the Exhibit Hall** (Sponsorship Available)

**5:20 BREAK-OUT DISCUSSIONS in the Exhibit Hall**

**6:20 Close of Day**

## THURSDAY, FEBRUARY 4

### ENGINEERING FOR DELIVERY

**8:25 AM Chairperson's Remarks**

*Tugrul Kararli, Ph.D., President & Founder, Pharmacricle*

**8:30 Tumor Targeting Theory-Kinetic & Diffusive Processes that Determine Antibody Macro & Microdistribution**

*K. Dane Wittrup, Ph.D., C.P. Dubbs Professor, Chemical Engineering & Biological Engineering, Associate Director, Koch Institute for Integrative Cancer Research, Massachusetts Institute of Technology*

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#### 9:30 Delivery of Antibodies – Market Analysis & Overview

Tugrul Kararli, Ph.D., President & Founder, Pharmacircle

#### 10:00 Sponsored Presentation (Sponsorship Opportunity Available)

#### 10:30 Poster Competition Refreshment Break & Raffles in the Exhibit Hall

### DELIVERY OF ANTIBODIES

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Anna M. Wu, Ph.D., Professor, David Geffen School of Medicine at UCLA

Cancer-targeting antibodies have been optimized for *in vivo* imaging by conversion into fragments such as diabodies, minibodies, and scFv-Fc. Recombinant fragments recognizing a variety of cell-surface markers have been labeled with positron-emitting radionuclides (I-124, Cu-64, F-18) for positron-emission tomography (PET) detection of tumors in preclinical models. ImmunoPET represents a broad platform for conducting “immunohistochemistry *in vivo*” to address biological questions in living organisms, including target expression, target coverage, and response to therapy.

#### 12:00 Advanced Polymer Conjugate Technology for Optimization of Cancer Therapeutics

Christine Loehrlein, Ph.D., A.D., New Products and Technology Strategy Research, Nektar Therapeutics

Conjugation of a therapeutic agent to polyethylene glycol and other polymers is a general strategy that can be used to optimize pharmacological parameters of that drug, with the ability to affect both its efficacy and side effect profile. Nektar's Advanced Polymer Conjugate Technology platform can be used to enable a wide range of molecules, including proteins, peptides, small molecule oral and parenteral drugs, and antibody fragments. Nektar is currently using this approach to develop advanced oncolytics with sustained exposure to tumor cells, and exploring opportunities to extend this technology to other cancer therapeutics.

#### 12:30 Luncheon Presentation (Sponsorship Opportunity Available) or Lunch on Your Own

#### 1:45 Ice Cream Refreshment Break in the Exhibit Hall

### PLENARY KEYNOTE SESSION

#### 2:15 Plenary Keynote Introduction

#### 2:25 Plenary Keynote Presentation (See Page 26 for Details)

#### 3:05 Refreshment Break in the Exhibit Hall

### CLINICAL CHALLENGES WITH RNAI THERAPEUTICS

#### 3:45 Chairperson's Remarks

Bob D. Brown, Ph.D., Senior Vice President, Research, Dicerna Pharmaceuticals Corp.

#### 3:50 LNA Antimirs – Pioneering microRNA Therapeutics

Henrik Orum, M.Sc., Ph.D., VP and CSO, Santaris Pharma

Short, single stranded LNA oligonucleotides delivered systemically as

naked molecules are able to potently and safely inhibit therapeutically attractive miRNAs in a range of tissues in experimental animals. The presentation will provide an update on the unique features of LNA oligonucleotides in miRNA therapeutics with particular emphasis on the pre-clinical and clinical development of SPC3649, an LNA AntimiR targeting miRNA-122.

#### 4:20 Pre-clinical and Clinical Development of Atu027 (siRNA-lipoplex/AtuPLEX) for Oncology

Klaus Giese, Ph.D., CSO, Silence Therapeutics plc

Atu027 refers to a liposomally formulated siRNA targeting PKN3 expression in the vascular endothelium. Pre-clinical studies in rodents and non-human primates demonstrated that intravenous administration is well tolerated and gives rise to RNAi-mediated suppression of PKN3 gene expression. Various proof-of-concept experiments on mouse tumor xenografts suggest profound inhibition of tumor progression and particularly of metastasis, which laid the foundation for therapeutic application in oncology. Atu027 is currently tested in a Phase-I clinical trial on subjects with advanced solid cancers. Pre-clinical data emphasizing pharmacological activity in mouse models and an update on the current Phase-I study will be discussed.

#### 4:50 Talk Title to be Announced

Ian MacLachlan, Ph.D., CSO, Tekmira Pharmaceuticals

#### 5:20 Talk Title to be Announced

Bob D. Brown, Ph.D., Senior Vice President, Research, Dicerna Pharmaceuticals Corp.

#### 5:50 Close of Day

### FRIDAY, FEBRUARY 5

### NOVEL FORMULATIONS FOR RNAI DELIVERY

#### 8:30 AM Chairperson's Opening Remarks

C. Sathishchandran, Ph.D., Chief Technology Officer, Research Technology Center, Pfizer, Inc.

#### 8:35 Therapeutic siRNA Delivery: Tackling the 800 Pound Gorilla

Steven F. Dowdy, Ph.D., Investigator, Howard Hughes Medical Institute; Professor, Department of Cellular & Molecular Medicine, University of California, San Diego School of Medicine

To date, siRNA delivery remains the rate-limiting step for RNAi therapeutics development. We developed a Peptide Transduction Domain-dsRNA Binding Domain (PTD-DRBD) fusion protein siRNA delivery approach. PTD-DRBD delivered siRNAs induced RNAi responses in the entire population of all cell types assayed (primary and tumorigenic) in a non-cytotoxic fashion. PTD-DRBD combinatorial delivery of EGFR and Akt2 siRNAs induced a synthetic lethal response that significantly increased survival of intracerebral glioblastoma pre-clinical models. These observations demonstrate the ability of PTD-DRBD to efficiently deliver siRNAs.

#### 9:05 Induction of Therapeutic Gene Silencing in Leukocyte-Implicated Diseases by Targeted and Stabilized Nanoparticles

Dan Peer, Ph.D., Head, Laboratory of Nanomedicine, Department of Cell Research and Immunology and the Center for Nanoscience and Nanotechnology, Tel Aviv University

Leukocytes are among the most difficult cells to transduce with RNAi. We developed a strategy that can target different subsets of leukocytes and selectively silence genes *in vivo* using targeted, stabilized nanoparticles (tsNPs). These carriers do not induce lymphocyte activation, interferon responses or release liver enzymes and are fully degradable. Three preclinical examples inflammatory bowel disease (IBD), blood cancer and viral infection will be discussed. We will show that tsNPs can be used for *in vivo* validation of new drug targets, for prevention of viral infection and for inducing therapeutic gene silencing in a preclinical setting.



## 9:35 Characterization of Immune Responses to tkRNAi Therapeutics

Johannes Fruehauf, M.D., Ph.D., VP, Research, Cequent Pharmaceuticals, Inc.

Transkingdom RNA interference (tkRNAi) describes a novel method for delivery of therapeutic RNA interference into gastrointestinal tissues using engineered bacteria which produce and deliver mediators of RNAi. Clinical trials are about to begin for the prevention of colon Polypoidosis, and for the treatment of Inflammatory Bowel Disease (IBD). Here we demonstrate recent results from large screening efforts characterizing the effects of tkRNAi on cytokine profiles and innate immunity.

## 10:05 Sponsored Presentation (Sponsorship Opportunity Available)

## 10:20 Coffee Break

## 11:00 Panel: Do We Understand the Challenges We Face With RNAi Therapeutics?

Panelists:

Bob D. Brown, Ph.D., Senior Vice President, Research, Dicerna Pharmaceuticals Corp.

Dmitry Samarsky, Ph.D., VP, Technology Development, RXi Pharmaceuticals

Ian MacLachlan, Ph.D., CSO, Tekmira Pharmaceuticals

John Rossi, Ph.D., Professor and Chair, Molecular Biology, Beckman Research Institute of the City of Hope

Steven Highlander, Ph.D., Partner, Intellectual Property, Fulbright & Jaworski, L.L.P.

## 12:00 PM Luncheon Presentation (Sponsorship Opportunity Available) or Lunch on Your Own

### NOVEL APPROACHES FOR TARGETED DELIVERY

## 1:00 Chairperson's Remarks

John Rossi, Ph.D., Professor and Chair, Molecular Biology, Beckman Research Institute of the City of Hope

## 1:05 *In vivo* delivery of Dicer substrate RNAs for treatment of HIV infection

John Rossi, Ph.D., Professor and Chair, Molecular Biology, Beckman Research Institute of the City of Hope

The application of RNAi for treatment of HIV infection has many advantages over conventional drugs. The inhibitors can be rapidly changed according to the viruses ability to mutate. This presentation will discuss the use of aptamers and dendrimers to deliver Dicer substrate RNAs *in vivo*. The results obtained demonstrate that Dicer substrate siRNAs can be delivered in a cell type specific manner with an aptamer, and can be generally delivered with dendrimers to effectively inhibit HIV replication in a humanized mouse model.

## 1:35 Targeted RNA-based Cancer Therapies

Paloma H. Giangrande, Ph.D., Assistant Professor, Department of Internal Medicine, University of Iowa

A major hurdle for the clinical translation of siRNAs into effective therapies is delivery. We describe an RNA aptamer-based approach for the targeted delivery of siRNAs to prostate cancer (PC) cells. The aptamer-siRNA reagent (chimera) is effective when administered systemically and is suitable for efficient chemical synthesis. When administered systemically to mice bearing PSMA-positive tumors, the RNA chimera triggered tumor regression without affecting normal tissues. This work is the first description of *in vivo* efficacy following systemic administration of an aptamer-siRNA chimera and thus represents a milestone for this platform technology.

## 2:05 Development of Novel Therapeutic RNAi Compounds and Effective *in vivo* Delivery Approaches

Dmitry Samarsky, Ph.D., VP, Technology Development, RXi Pharmaceuticals

RNA interference (RNAi) offers a novel approach to the drug development process, because RNAi compounds can potentially be designed to target any one of the genes in the human genome. Other potential advantages of RNAi therapeutics include, rapid development of lead compounds, high selectivity for the target gene, high potency (low dose) and low toxicity due to natural mechanism of action. We will introduce unique single-oligo (rxRNA<sup>solo</sup>) and short-duplex (rxRNA<sup>nano</sup>) RNAi compounds, as well as novel *in vivo* delivery approaches, including self-delivering rxRNA molecules (sd-rxRNA) for local and systemic delivery, and targeted delivery to phagocytic immune cells using.

## 2:35 Talk Title to be Announced

C. Satishchandran, Ph.D., Chief Technology Officer, Research Technology Center, Pfizer, Inc.

## 3:05 Close of Conference

## Cambridge Healthtech Institute's Sixth Annual Translational Medicine Completing the Circle: Bedside Back to Bench

WEDNESDAY, FEBRUARY 3

**7:00 AM Registration and Morning Coffee**

**8:00 Plenary Keynote Session** (See Page 26 for Details)

**9:40 Grand Opening Refreshment Break in the Exhibit Hall**  
**TRENDS IN TRANSLATIONAL MEDICINE**

**11:00 Chairperson's Remarks**

Christina M. Coughlin, M.D., Ph.D.; Medical Director, Oncology; Clinical Research and Development; Pfizer Oncology

**11:10 Translational Approach to Studying Stroke**

Giora Z. Feuerstein, M.D., Assistant VP, Discovery Translational Medicine, Wyeth Research Labs

To improve success of clinical trials and speed drug development, departments of Translational Medicine in pharma have formed to figure out the which molecular, biochemical and physiological biomarkers can best substitute for the absence of clinical outcome studies. My presentation will illustrate how we've applied a translational approach to develop better therapies for stroke. Specifically we've focused on reducing attrition rate of compounds/biologicals by optimizing 1. Target Validation; 2. Compound-Target interaction; 3. innovative Pharmacokinetic-Pharmacodynamic and proof of Mechanism of Action (MoA); 4. disease biomarkers; 5. Patient selections for clinical trials based on evidence for likelihood to respond to treatment.

**11:40 Contribution of Translational Approaches to Recent**

**Advances in Immuno-therapeutics, Immuno-rejection and Beyond**  
Francesco Marincola, M.D., Chief, Infectious Disease and Immunogenetics Section, NIH; Editor in Chief, Journal of Translational Medicine

The complexity underlying a pathological process does not necessarily require complex solutions. The biology determining allograft or cancer rejection, autoimmunity or tissue damage during pathogen infections is complex; however, common patterns are emerging that lead to a common final outcome: tissue destruction with resolution of the pathogenic process (cancer, infection) or tissue damage and organ failure (allograft rejection, autoimmunity). Human observations based on transcriptional profiling converge into an "immunological constant of rejection" that signals such occurrences. This constant includes the coordinate activation of interferon stimulated genes (ISGs) and immune effector functions (IEFs). Understanding this final effector pathway may suggest novel strategies for the induction or inhibition of tissue-specific destruction with therapeutic intent in cancer and other immune pathologies. This presentation will discuss how vaccines may play a role in tissue-specific destruction and use this as a model to demonstrate how to understand the dynamics of therapeutics by studying target tissues in real time.

**12:10 PM Panel: Practical Translational Medicine**

Moderator: Vivek Kadambi, Ph.D., Senior Director, Millenium Pharmaceuticals

- When to use biomarkers for go/no-go decisions on proceeding with development of a clinical compound
- How has translational medicine changed over the past 5 years?
- Fostering partnerships between industry, academics and government granting institutions
- How are pharmacodynamic and predictive markers being used right now in clinical development?

Panelists: Giora Z. Feuerstein, M.D., Assistant Vice President and Head, Discovery

Translational Medicine, Wyeth Research Labs

Lise Kjems, M.D., Ph.D., Executive Director, Global Program Diagnostic Director, Molecular Diagnostics Novartis Francesco Marincola, M.D., Chief, Infectious Disease and Immunogenetics Section, NIH; Editor-in-Chief, Journal of Translational Medicine William B. Mattes, PhD, DABT, PharmPoint Consulting, Former Executive Director, Predictive Safety Testing Consortium

Yali Fu, Ph.D., Program Director, Grants and Contracts Operations Branch, Developmental Therapeutics Program, Division of Cancer Treatment and Diagnosis, National Cancer Institute

Hans Winkler, Ph.D., Senior Director, Global Head Oncology Biomarkers, Johnson & Johnson

**12:40 Luncheon Presentation I**

Sponsored by  PreClinOmics

**ZSD Rat: A Model for Diabetes, Metabolic Syndrome, and Obesity without Leptin or Leptin Receptor Mutations**

Richard G. Peterson, Ph.D.; Executive VP Research & Development, PreClinOmics, Inc.

- The ZSD rat is a model for obesity, insulin resistance, metabolic syndrome and diabetes with a normal leptin axis.
- The disease conditions expressed in the ZSD rat more closely resemble the human situation when compared to other animal models.
- The obesity and diabetes in the ZSD rat can be modulated with diet.
- The obesity and diabetes in the ZSD rat can be treated with standard pharmaceuticals.
- The ZSD rat expresses the bone, renal and other complications seen in diabetes.

**1:10 Luncheon Presentation II** (Sponsorship Opportunity Available)

**1:45 Dessert in the Exhibit Hall**

**WORKING BACKWARDS IN CANCER:  
FROM THE CLINIC TO DISCOVERY**

**2:15 Chairperson's Remarks**

Michael Lieberman, Ph.D., Managing Director, Strategic Medicine, Inc.

**2:20 Using Drug-Induced Feedback Loops to Identify Indications and Combination Partners**

Donald Bergstrom, Ph.D., Director, Experimental Medicine Oncology, Merck

James W. Watters, Ph.D., Associate Director, Molecular Profiling Oncology, Merck

Treatment with molecular targeted agents can result in compensatory feedback regulation as cells respond to inhibition of signaling pathways. We will present clinical evidence that treatment with a small molecule inhibitor of gamma secretase results in pathway modulation and compensatory feedback, and describe pre-clinical experiments designed to leverage this concept for drug response prediction.

**3:00 Moving Research Closer to the Bedside, *in vitro* and *in vivo* Analyses with Primary Tumors**

Fred Poordad, M.D., Chief of Hepatology, Liver Disease and Transplant Center, Cedars-Sinai Medical Center, Xin Wei Wang, Ph.D., Senior Investigator Head, Liver

Carcinogenesis Section, Laboratory of Human Carcinogenesis, National Cancer Institute, NIH, and Michael R. Briggs, Ph.D., Senior Director, Biology, Vertex Pharmaceuticals, Inc.

The incidence of Primary Liver Cancer is increasing in the west and constitutes a tremendous burden on world health as the third leading cause of cancer deaths worldwide. The 5 year survival rate is a dismal 11 %, due in large part to late diagnosis and limited treatment options. The etiology of this devastating disease as well as current and proposed new therapies will be discussed. Steps to better diagnose and stratify patients for targeted therapy will be considered as a new and exciting phase of cancer research. Finally, a move toward more relevant research will be presented as an hypothesis that will be tested in the coming years as more new and current therapies are compared and contrasted to current best practice.

#### 4:05 How do you go from Pathways to Clinical Outcomes?

Aris Persidis, Ph.D., President, Biovista, Inc.

In drug discovery and development what really counts is the clinical outcome, the Benefit/Risk of the drug within the context of its pathway or mechanism of action (MoA). Biovista screens the MoA of any drug or target against the MoA of 8,000 indications and 12,000 adverse events (AEs). This is simultaneous, unbiased indication discovery and AE profiling, and it is unique. It helps to bridge the gap from molecular pathway to clinical outcome in a single step. Case studies in cancer and other diseases will be described.

#### 4:35 Reception in the Exhibit Hall (Sponsorship Available)

#### 5:20 BREAK-OUT DISCUSSIONS in the Exhibit Hall

##### 1. Novel Imaging Biomarkers in Drug Development

Moderator: Jingsong Wang, M.D., Director of Immunology, Discovery Medicine & Clinical Pharmacology, Bristol-Myers Squibb, Co.

- The distinct advantage and unique challenges in applying imaging biomarkers in drug development
- The most promising novel imaging biomarkers for drug development, and which therapeutic areas will benefit the most from using imaging biomarker
- The role of pharmaceutical company, CRO, academia and regulatory agency in the discovery, development and qualification of imaging biomarkers

##### 2. Biomarkers in Translational Medicine

Co-Moderators:

Christina M. Coughlin, M.D., Ph.D.; Medical Director, Oncology; Clinical Research and Development; Pfizer Oncology

Hans Winkler, Ph.D., Senior Director, Global Head Oncology Biomarkers, Johnson & Johnson

- Which type of biomarkers will speed drug development the most?
- Predictive marker clinical validation: retrospective vs. prospective trial requirements
- Do we still need pharma dynamic analysis in Phase II?

##### 3. New Funding Opportunities for Biotechs

Moderator:

Yali Fu, Ph.D., Program Director, Grants and Contracts Operations Branch, Developmental Therapeutics Program, Division of Cancer Treatment and Diagnosis, National Cancer Institute

- Understand NIH Enhanced Peer Review
- NCI's new initiatives on SBIR funding
- Phase II Bridge awards to help biotechs further develop their technologies

##### 4. New Animal Models in Translational Medicine

- Ideal characteristics for new animal models
- Advantages of new animal models
- Designing studies and using new animal models in discovery research

Co-Moderators:

Richard G. Peterson, Ph.D., Professor Emeritus, Indiana University School of Medicine and EVP, Research and Development PreClinOmics, Inc.

Troy A. Gobbett, MS, Director, Sales & Marketing for PreClinOmics, Inc.

#### 6:20 Close of Day

### THURSDAY, FEBRUARY 4

#### IMAGING: CLINICAL TO PRE-CLINICAL

##### 8:25 AM Chairperson's Remarks

Jingsong Wang, M.D., Director, Immunology, Discovery Medicine & Clinical Pharmacology, Bristol-Myers Squibb, Co.

##### 8:30 Assessing Mechanism of Action of Anticancer Agents using Functional Imaging in Oncology Drug Development

Dana Hu-Lowe, Ph.D., Group Leader, Associate Research Fellow, Cancer Biology, Pfizer, Inc., PGRD-La Jolla

Multiple imaging modalities have helped us to gain a deeper understanding of the mechanism of action of drug candidates beyond conventional pharmacological end points used in nonclinical and clinical settings. More importantly, functional imaging modalities are providing in-depth information on the modes of action of various anti-angiogenesis agents. These learnings are vital for improving efficiency in drug development.

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#### 9:00 Animal Imaging for Translational Approaches to CNS

##### Drug Discovery

Rudy Schreiber, Ph.D., Senior Director, Pharmacology, Discovery and Early Clinical Research

In this presentation, I demonstrate how Sepracor's translational medicine approach for CNS drug discovery using emerging imaging methods and collaborative approaches such as cooperative technology development within a pain consortium. I'll present animal SPECT data for our inhibitors of monoamine neurotransmitter transporters (serotonin, noradrenaline and dopamine) in rodents and non human primates (with one example of human PET): I will include also brain microdialysis data in rodents where we measured all 3 neurotransmitters, and share human data generated in a fMRI pain imaging consortium.

#### 9:30 Image Analysis Considerations for Pre-clinical, *in vivo* Medical Imaging

Matthew Silva, Ph.D., Head, Imaging Sciences, Millennium, The Takeda Oncology Company

#### 10:00 Standardized Solutions for Non-Invasive Imaging of Cell Trafficking

Eric T. Ahrens, Ph.D., Founder and Chief Scientific Officer, Celsense, Inc.

A limiting factor in the development of new therapies is an inability to non-invasively assay cell trafficking *in vivo*. In this talk Dr. Eric Ahrens will describe a unique non-invasive imaging platform for visualizing and quantifying cells *in vivo* using magnetic resonance techniques. Applications include visualization and quantification of transplanted cells in immunotherapy and regenerative medicine and monitoring of inflammatory processes.

#### 10:30 Poster Competition Refreshment Break & Raffles in the Exhibit Hall

### CHALLENGES IN TRANSLATIONAL BIOMARKER DEVELOPMENT

#### 11:30 Biomarkers for Proof of Concept and Patient Selection

Hans Winkler, Ph.D., Senior Director, Global Head Oncology Biomarkers, Johnson & Johnson

The talk will comprise a discussion about the value of pharmacodynamic markers for proof of principle and decision making in Phase I/II. Furthermore, the necessity and utility of predictive markers that can be applied for patient selection will be discussed.

#### 12:00 PM Patient Selection Biomarker Approaches in Oncology

Daniel S. Johnston, Ph.D., Principal Research Scientist II, Pfizer Research and Development

This presentation will focus on the current strategies to identify patient selection biomarkers in oncology. Both clinical and preclinical approaches will be discussed.

#### 12:30 Luncheon Presentation (Sponsorship Opportunity Available) or Lunch on Your Own

#### 1:45 Ice Cream Refreshment Break in the Exhibit Hall

### PLENARY KEYNOTE SESSION

#### 2:15 Plenary Keynote Introduction

#### 2:25 Plenary Keynote Presentation (See Page 26 for Details)

#### 3:05 Refreshment Break in the Exhibit Hall

### REDESIGNED ANIMAL MODELS

#### 3:45 Chairperson's Remarks

Alain Stricker-Krongrad, Ph.D., Senior Scientific Adviser, Global Business Development, Charles River

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### 3:50 An Animal Model of Parkinson's Disease Psychosis: Assessing Potential Therapeutic Efficacy of 5-HT<sub>2A</sub> Inverse Agonists

Krista McFarland, Ph.D., Team Leader, In Vivo Pharmacology, ACADIA Pharmaceuticals, Inc.

Available antipsychotic drugs do not provide an ideal treatment for psychosis in Parkinson's disease (PDP) because their blockade of dopamine receptors counteracts the dopamine replacement therapy used to alleviate the motor symptoms of PD. Development of alternate pharmacotherapies is limited by the lack of an animal model. Recent efforts to develop such a model and assess the potential therapeutic efficacy of 5-HT<sub>2A</sub> inverse agonists for the treatment of PDP will be discussed.

### 4:20 Engineered Human-In-Mouse Tumors for Population Based *in vivo* Biomarker Discovery

Min Wu, Ph.D., Principal Scientist, Translational Research, AVEO Pharmaceuticals, Inc.

I will present a NEW population-based tumor model system using Human-in-Mouse tissue transgenic human tumors that feature naturally occurring tumor variation akin to that observed in human tumor populations. The goal is to identify and validate biomarkers that ultimately predict responsive versus non-responsive patient populations to guide clinical development.

### 4:50 Appropriate Animals Models for Safety Assessment of Biologics

Timothy MacLachlan, Ph.D., Associate Director of Nonclinical Safety Assessment, Genzyme Corporation

The proper safety assessment of biopharmaceuticals has been an evolving process. While the paradigm set for small molecules was applied early, and some aspects have remained the same, other areas have required modification. The specificity of some biologics like monoclonal antibodies has necessitated study in higher order species. However, alternatives such as mice transgenic for the human target have proved useful, and at times, more accurate in risk assessment. Examples of these approaches will be discussed.

### 5:20 Comparative Oncology Drug Development

Melissa C. Paoloni, DVM, DACVIM, Director, Comparative Oncology Program, National Institutes of Health, National Cancer Institute

Comparative oncology is a model system to evaluate novel drugs, devices, biologics and imaging strategies in pet dogs with cancer to help inform their development for human cancer patients. Although much of this effort is preclinical, it also applies to agents that are already "first in man." The goal behind it is to garner information to help make informed go and no-go decisions to drive human oncology clinical trial design by answering questions about PK, PD, schedule, regime, dose, toxicity and clinical outcome (to just name a few). It has been well recognized and utilized by many within the pharmaceutical industry. It also has the ability to pilot personalized medicine approaches-a key to the future of oncology drug development.

### 5:50 Close of Day

FRIDAY, FEBRUARY 5

## TRANSLATIONAL INFORMATICS – HOW FAR HAVE WE COME?

### 8:30 AM Chairperson's Opening Remarks

Pearl S. Huang, Ph.D., Integrator, Oncology Franchise, Research & Early Development, Merck & Co.

### 8:35 Implementing a Translational Biomarker Strategy to Reduce Attrition in Drug Development

Irina Antonijevic, M.D. Ph.D., Director, Translational Research, Biological Research, Lundbeck Research, Inc. USA

Early efforts towards the discovery of molecular biomarkers for CNS disorders are encouraging. However, confirmation, and ultimately vali-

dation of such biomarkers is dependent on state-of-the-art bioinformatics analyses as well as assay development. These prerequisites will ensure identification of biomarkers that are reproducible and hence of clinical relevance.

### 9:05 High Content Mining of Disease Biomarkers

Jake Chen, Ph.D., Assistant Professor, Informatics & Computer Science, Indiana University School of Informatics; Director, Indiana Center for Systems Biology and Personalized Medicine, Indiana University - Purdue University Indianapolis; Founder, MedeoLinx, Inc.

To facilitate the interpretation of raw Omics data into detailed disease-specific knowledge of candidate biomarkers, we developed a "high-content biomarker mining" software system. The system can help manage and correlate molecular functions, molecular connectivity, biological pathways, and literature information. Its application into the current biomarker development process will help improve the success rate and quality of candidate biomarkers.

### 9:35 Single Molecule Real Time Biology: New technologies Enabling a More Complete Characterization of Disease Biology

Eric Schadt, Ph.D., Chief Scientific Officer, Pacific Biosciences

While there has been an explosion of technologies that enable more comprehensive characterizations of complex biological processes like common human diseases, we are still unable to glimpse a large enough fraction of the biology of these systems to build models that are predictive enough to achieve clinical utility. However, with a new wave of technologies on the horizon, providing for the capability to examine the activity of single molecules real time, we will soon be capable of generating the right scale and diversity of data (DNA sequence, RNA sequence, real time monitoring of mRNA translation, full characterizations of base modifications in genomes and transcriptomes) at low cost to dramatically enhance the construction of models for common human diseases that achieve clinical utility. I will cover the single molecule real time (SMRT) technologies from Pacific Biosciences and how these technologies will revolutionize our ability to characterize living systems, and then present a number of integrative biology approaches to taking the types of data SMRT technologies will generate to get at predictive models of disease that can be used to drive the identification and validation of drug targets and biomarkers.

### 10:05 Automating Biomarker Discovery and Qualification; Capturing Hypothesis, Analysis and IP

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Jonathan Sheldon, Ph.D., Director of Translational Medicine, IDBS

Long lists of un-annotated proteins and genes are not a sufficient end point for 'omics analysis, they need to be annotated with data from many public and proprietary sources. IDBS provide solutions to not only automate the discovery and subsequent annotation of biomarker results, but to capture each step of the experimental set up, data capture, and analysis in a compliant manner.

### 10:20 Coffee Break

## TRANSLATIONAL INFORMATICS – HOW FAR HAVE WE COME? (CONTINUED)

### 11:00 Profiling Patients to Drive Biomarker Development

N. R. Nirmala, Ph.D., Director, Biomarker Analysis and Informatics Unit, Translational Sciences, Novartis Institutes of Biomedical Research

Gene expression profiling is one of the key ways in which a genome-wide view of a patient's response to drug treatment can be obtained. Such a molecular level view can provide strategies for customized therapies in many contexts. In this talk, the opportunities and challenges that this technology presents will be discussed with a couple of case studies. Extension of this approach to other technologies will also be presented in the context of biomarker development.

### 11:30 Panel: Informatics at R&D Interphases

Moderator: Pearl S. Huang, Ph.D., Integrator, Oncology Franchise, Research & Early Development, Merck & Co.

- Linking clinical outcome with molecular data: filling the gaps
- Capturing uniform clinical language for outcomes
- Compatible and user- friendly data systems—can one size fit all?
- Disease cohorts-how many, how big, what is acceptable quality

**12:00 PM Luncheon Presentation** (Sponsorship Opportunity Available) **or Lunch on Your Own**

## CASE STUDIES ON SUCCESSFUL ORGANIZATIONAL COLLABORATIONS AND SYSTEM APPROACHES

### 1:00 Chairperson's Remarks

William B. Mattes, Ph.D., DABT, Former Executive Director, Predictive Safety Testing Consortium, Critical Path Institute

### 1:05 Taking personalized medicine to the next level- The critical Interface between Translational Medicine and Molecular Diagnostics

Lise Kjems, M.D. Ph.D., Executive Director, Global Program DiagnosticDirector, Molecular Diagnostics Novartis

Translational Medicine has reformed our drug development paradigm. Early clinical profiling of New Molecular Entities can enable identification of subsets of patients with a unique risk/benefit profile. In this talk, the opportunities and challenges related to this identification will be discussed. The role of Molecular Diagnostics is critical in developing and integrating novel patient treatment algorithms

### 1:35 Fostering Collaborations between Biotech and Academics to Speed Translational Medicine

Thomas Ichim, Ph.D., CEO, MediStem Labs, Inc.

### 2:05 Commercial Collaborations and other Approaches to Direct Academic Cancer Research towards Clinical Outcomes

Clive Stanway, Ph.D., CSO, Cancer Research Technology Ltd., Wolfson Institute for Biomedical Research

Cancer Research Technology (CRT) is the development and commercialization arm of Cancer Research UK (CR-UK) which has an annual science spend in excess of \$500M. CRT works with CR-UK through multiple tracks to drive translational research including dedicated industry experienced, peer-reviewed funding for managed research in the PI's laboratory or in collaboration with focused drug discovery research groups around the UK. Specific examples and outcomes of this strategy will be presented with some discussion of CRT's flexible and creative approach to partnerships.

### 2:35 Development of Combination Therapies for Multiple Sclerosis Using Systems Level Informatics

Frederic S. Young, Ph.D., Chief Scientist, Vicus Therapeutics

We start with a multilevel systems physiology model that combines metabolomic analysis with integrated physiological analysis. The model is used to define a set of systems informatic features of ontogeny, phylogeny, homeostasis, and repair that distinguishes the disease state from homeostasis. We describe our use of this systems informatic signature as an algorithm for the development of combination therapies for multiple sclerosis.

**3:05 Close of Conference**



# PLENARY KEYNOTE PRESENTATIONS

**WEDNESDAY, FEBRUARY 3**

## 8:10 - 8:55 am When Drug Research is Personal



*John F. Crowley, Founder, Novazyme Pharmaceuticals, Inc.*

Mr. Crowley's emotion-packed presentation will focus on his personal struggle to find a cure for Pompe disease, a rare and fatal illness that is caused by a defective or missing enzyme. Pompe disease affects fewer than 10,000 people world-wide, including Mr. Crowley's two small children.

Mr. Crowley, a Harvard educated businessman, created and built a pharmaceutical company devoted expressly to finding a cure for the disease. He will detail his journey through the labyrinth of scientific and business fronts, which lead up to a first-round clinical trial.

## 8:55 - 9:40 am Technology, Aging, and the Brain



*Gary W. Small, M.D., Parlow-Solomon Professor on Aging, Professor of Psychiatry & Biobehavioral Sciences, Director, UCLA Center on Aging, Director, Memory & Aging Research Center, Director, Geriatric Psychiatry Division, Semel Institute for Neuroscience & Human Behavior, David Geffen School of Medicine at UCLA*

New neuroimaging and other technologies are teaching us about how the brain ages and what we can do about it. Although memory declines as we age, medical and non-pharmacological strategies may protect brain health and improve memory performance. At the same time, innovation in digital technology is not only changing the way we live and communicate, it appears to be altering how our brains function. As a consequence of this high-tech stimulation, we are witnessing the beginning of a new form of the generation gap – a *brain gap* dividing younger digital natives, immersed in the technology early in life, from older *digital immigrants*, who adapt to the new technology more reluctantly. This lecture will describe this current pivotal point in brain evolution and how we can harness the new technology and lifestyle choices to improve memory and brain function so we can live better and longer.

**THURSDAY, FEBRUARY 4**

## 2:25 - 3:05 pm Chips, Clones and Living Beyond 100



*Paul J.H. Schoemaker, Ph.D., M.B.A., Chairman and Chief Executive Officer, Decision Strategies International, Inc.; Research Director, Mack Center for Technological Innovation, The Wharton School; Adjunct Professor of Marketing, The Wharton School Adjunct Professor, Wharton School of Business*

As information technologies and life sciences continue to converge, new business opportunities and challenges will arise for the field of diagnostics and beyond. This keynote reviews the deeper forces shaping the future of the biosciences, from social and economic to technological and political, including the stresses they will introduce for existing business models and healthcare. Not only will bio-convergence introduce new products, services and competitors, it may create entirely new industries on a scale larger than the computer revolution has to date. Several broad scenarios will be painted for the state of the biosciences in 2025 and the forces that might take us there, summarizing a multi-year strategy study conducted and supervised by the speaker at the Wharton school.

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### Basic Immersion Topics Include:

#### Learning Objectives:

- Gain a fundamental understanding of the science and technology driving the Biotech/Pharma industry
- Learn basic scientific terminology used by researchers in the life sciences

#### Industry Overview

- Industry Sectors

#### The Science:

- DNA
- Genetic Variation
- Proteins
- Genetic Basis of Disease

#### The Technology:

- Restriction Enzymes
- Recombinant DNA
- Recombinant Proteins
- Plasmids
- Biologics

#### Drug Discovery:

- Finding a Target
- Designing a Targeting Strategy
- Assay Development
- Pharmacogenomics

#### Drug Development:

- Preclinical Trials
- Clinical Trials
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- University administrators, research institute support staff

### Course Instructor



*Karin Lucas, Ph.D., Biotech Primer Instructor and Scientific Advisor*

*Karin Lucas, Ph.D., BioTech Primer Instructor and Scientific Advisor*

Karin Lucas, Ph.D., has been teaching with BioTech Primer, Inc. for the past five years. As a scientist at Biogen Idec she develops protein pharmaceuticals for the treatment of cancer and multiple sclerosis. Previously, Dr. Lucas was a scientist and project director at Cardinal Health where she worked on the development of over 25 products with multiple pharmaceutical and biotechnology companies. In addition to her laboratory role, she is also trained as a Lean Six Sigma greenbelt. Dr. Lucas is an active community volunteer and has served as the PR chair and later Vice President of the San Diego chapter of AWIS (Association for Women in Science). In 1998, Dr. Lucas was honored as the Cal Poly Physical Chemistry Student of the Year and in 2003 she was selected for the AWIS San Diego Rookie of the Year Award. Dr. Lucas received her B.S. in biochemistry from California Polytechnic State University, San Luis Obispo and went on to complete her Ph.D. in biochemistry at University of California San Diego.

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- ☐ SC4 Addressing Safety Concerns For Biological Drugs
- ☐ SC5 Targeting Cancer Stem Cells With Biologics
- ☐ SC6 Blood-Brain Barrier

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- ☐ SC7 Best Practices In Translational & Personalized Medicine
- ☐ SC8 Strategies for Molecular Diagnostic Companies
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- ☐ Cancer Profiling and Pathways

#### BIOLOGICS CHANNEL

- ☐ Stem Cells
- ☐ RNA Interference
- ☐ Cancer Biologics
- ☐ Delivery of Biologics

#### CANCER CHANNEL

- ☐ Cancer Biologics
- ☐ Cancer Molecular Markers
- ☐ Cancer Profiling and Pathways

#### EXECUTIVE CHANNEL

- ☐ Translational Medicine

#### DISCOUNTS\*

☐ Poster (\$50 off)

☐ Alumni (20% off)

☐ **BAYBIO** (10% off)

☐ Hotel (\$75 off) see pg 18 for details

Hotel Confirmation number is \_\_\_\_\_

\*Alumni and Bay Bio Discount cannot be combined. Discounts not applicable on Pre-Conference Events, (Conference registrations only)

- ☐ I cannot attend but would like to purchase the Molecular Medicine Tri-Conference CD for \$750 (plus shipping). Massachusetts delivery will include sales tax

### PAYMENT INFORMATION

☐ Enclosed is a check or money order payable to Cambridge Healthtech Institute, drawn on a U.S. bank, in U.S. currency.

☐ Invoice me, but reserve my space with credit card information listed below.

Invoices unpaid two weeks prior to conference will be billed to credit card at full registration rate. Invoices must be paid in full and checks received by the deadline date to retain registration discount. If you plan to register on site, please check with CHI beforehand for space availability.

☐ Please charge: ☐ AMEX (15 digits) ☐ Visa (13-16 digits) ☐ MasterCard (16 digits)

Card # \_\_\_\_\_

Expiration Date \_\_\_\_\_

Cardholder \_\_\_\_\_

Signature \_\_\_\_\_

Cardholder's Address (if different from above) \_\_\_\_\_

City/State/Postal Code \_\_\_\_\_

Country \_\_\_\_\_

### Present a Poster and Save \$50!

Cambridge Healthtech Institute encourages attendees to gain further exposure by presenting their work in the poster sessions.

Special poster deadlines apply. To secure a poster board and inclusion in specific conference materials, your abstract must be submitted, approved and your registration paid in full by the following deadlines:

#### December 23, 2009

Poster abstracts submitted and approved by December 23, 2009 will be included in the Program Guide and Conference Proceedings Link.

#### January 8, 2010

Poster abstracts submitted and approved between December 24, 2009 and January 8, 2010 will not be included in the Program Guide, but instead will be included in the Program Addendum and Conference Proceedings Link.

All poster abstracts are due no later than **January 8, 2010**. Register online, or by phone, fax or mail. Indicate that you would like to present a poster and you will receive abstract submission instructions via email.

☐ Yes, I am interested in presenting a poster at:

**Molecular Medicine Tri-Conference**

Please refer to the Registration Code below:



Yes! I would like to receive a FREE  
eNewsletter subscription to:  
[www.chimedialogroup.com](http://www.chimedialogroup.com)

#### ☐ Weekly Update

The latest industry news, commentary and highlights from Bio+IT World

#### ☐ eCliniqua

Innovative management in clinical trials

#### ☐ Pharma Services News

Service solutions for discovery, pre-clinical and clinical trials

#### Additional Registration Details

Each registration includes all conference sessions, posters and exhibits, food functions, and a copy of the conference proceedings link.

#### REGISTER 3 - 4th IS FREE

Individuals must register for the same conference or conference combination and submit completed registration form together for discount to apply. Please reproduce this registration form as needed.

#### Group Discounts

Special rates are available for multiple attendees from the same organization. **Contact David Cunningham at 781-972-5472** to discuss your options and take advantage of the savings.



#### Handicapped Equal Access

In accordance with the ADA, Cambridge Healthtech Institute is pleased to arrange special accommodations for attendees with special needs. All requests for such assistance must be submitted in writing to CHI at least 30 days prior to the start of the meeting.

#### Substitution/Cancellation Policy

In the event that you need to cancel a registration, you may:

- Transfer your registration to a colleague within your organization.
- Credit your registration to another Cambridge Healthtech Institute program.
- Request a refund minus a \$100 processing fee per conference.
- Request a refund minus the cost (\$750) of ordering a copy of the CD.

NOTE: Cancellations will only be accepted up to two weeks prior to the conference. Program and speakers are subject to change.

#### CHI Insight Pharma Reports

A series of diverse reports designed to keep life science professionals informed of the salient trends in pharmaceutical technology, business, clinical development, and therapeutic disease markets. For a detailed list of reports, visit [InsightPharmaReports.com](http://InsightPharmaReports.com), or contact Rose LaRaia, [rlaraia@healthtech.com](mailto:rlaraia@healthtech.com), 781-972-5444.

#### Barnett Educational Services

Barnett is a recognized leader in clinical education, training, and reference guides for life science professionals involved in the drug development process. For more information, visit [www.barnettinternational.com](http://www.barnettinternational.com).

Video and/or audio recording of any kind is prohibited onsite at all CHI events.

**MED001722**



# ACCP

AMERICAN COLLEGE OF CLINICAL PHARMACOLOGY

*Advancing Clinical Care through Pharmacology®*

**2016 Annual Meeting**  
American College of  
Clinical Pharmacology

## **Clinical Pharmacology:**

*Discovery and  
Application  
in the Era of  
Precision Medicine*

**September 25 – 27, 2016**

Bethesda N Marriott Hotel & Conference Ctr, Bethesda, MD

Co-chairs: Vikram Arya, PhD, Honghui Zhou, PhD and Manish Gupta, PhD

**FINAL PROGRAM**

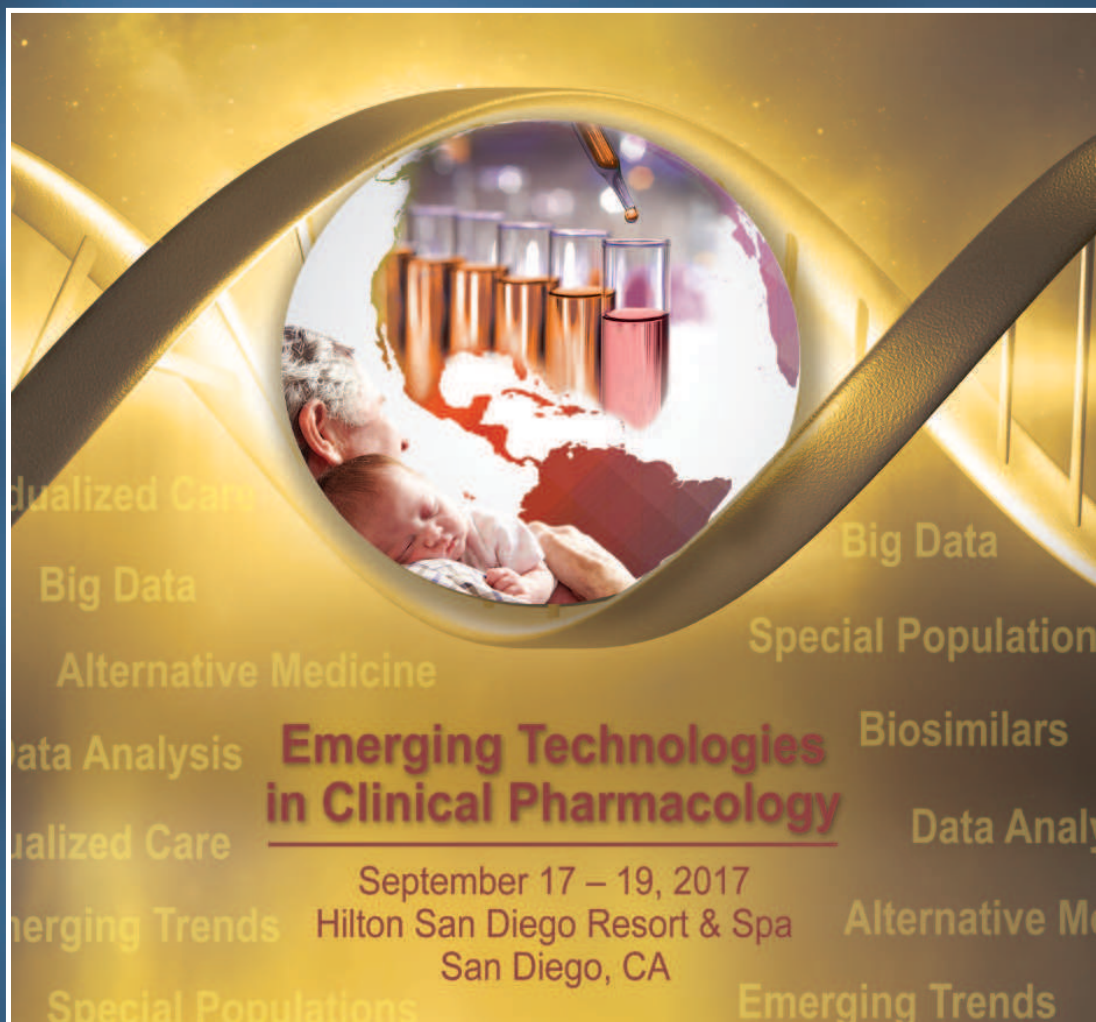
**MED001723**



# ACCP

AMERICAN COLLEGE OF CLINICAL PHARMACOLOGY  
Advancing Clinical Care through Pharmacology®

## Join Us for the **2017 ACCP Annual Meeting!**



ACCP is a proud provider of Continuing Medical Education (CME) & Continuing Pharmacy Education (CPE)

### **FUTURE MEETINGS:**

#### **2018 Annual Meeting**

September 23 – 25, 2018

Bethesda N Marriott Hotel & Conference Ctr

Bethesda, MD

## Did You Know?

### Use the ACCP Mobile App to find all the meeting information you're looking for!

- Session schedules organized by each day and track, with search functionality
- Session details including times, locations, session description, Faculty profiles and presentations
- Presentations from all sessions available real time and archival viewing (.pdf format) during and after the event
- Access attendee and Faculty lists, with search functionality
- Establish contact and network with fellow attendees
- Chat/instant message with fellow attendees
- View profiles of Faculty, ACCP Staff, Exhibitors & Sponsors with contact information
- Customize profile and business card on mobile device and/or import LinkedIn profile

### ACCP continues to expand its Continuing Education Program!

An accredited provider of Continuing Medical Education (CME) and Continuing Pharmacy Education (CPE), ACCP continues to expand its Continuing Education Program. In 2016, ACCP provided Journal CE articles each month and continued the ACCP Virtual Journal Club and Fundamentals Tutorials webinars, in addition to launching the Therapeutic Dilemmas webinars. Each of the live webinars is then made available On Demand. See page 13 for more information.



Virtual Journal Club

### Publish Your Manuscript in *The Journal of Clinical Pharmacology*

- The average time from submission to first decision is 14 days
- For manuscripts that are revised, the average time from the first submission to final decision is 25 days
- Downloads from JCP have increased by approximately 150% since 2013
- With the JCP App you can read the Journal anytime on your smart phone or tablet. Download this from the Apple App store.

### *Clinical Pharmacology in Drug Development* is now indexed by MEDLINE® and SCIE

#### Why publish with CPDD?

- Well respected: Official Journal of the ACCP
- A renowned international Editorial Board
- Growing international readership: full text article downloads increased by >38% in 2015!
- Quick and easy online submission and review process
- Rapid publication: articles are available online weeks ahead of issue publication

- Open access options available for authors who wish to make their article free for all to access online
- Immediate international exposure with PubMed/MEDLINE®, Web of Science: Science Citation Index Expanded (SCIE), Scopus, Chemical Abstracts, Embase and Google Scholar indexation

### Attending the Meeting as a Student or Trainee?


ACCP has planned a series of events specifically to benefit Students & Trainees! See page 43 for details.

### Interested in joining ACCP?

Stop by the ACCP Registration Desk for complete information or to complete a profile and pay 2017 Dues entitling you to ACCP Member Benefits.

### Take Time to Visit Our Exhibitors!

Exhibitor support is critical to the success of the ACCP Annual Meeting. We encourage you to visit our Exhibitors in Ballroom Salon E-H during breakfast, breaks or the evening receptions to learn about new technologies and service offerings. These exceptional Exhibitors are the leaders in their fields and are anxious to share with you the latest information on how they can help you meet your goals! Please take a moment to thank them for their support. All attendees are invited to participate in the Exhibit Hall Contest to win one of two \$50 gift cards by getting your game card stamped by all the Exhibitors. Game cards are provided in attendee tote bags.

“Like” ACCP and add to “My Page's Favorites” on Facebook  or join ACCP's **Linked in** Group for regular updates.

### ACCP Registration Desk Hours / Grand Foyer

Friday, September 23 <sup>rd</sup>	4:00 – 7:00 pm	Grand Foyer
Saturday, September 24 <sup>th</sup>	7:00 am – 5:30 pm	Grand Foyer
Sunday, September 25 <sup>th</sup>	6:30 am – 7:00 pm	Grand Foyer
Monday, September 26 <sup>th</sup>	7:00 am – 7:00 pm	Grand Foyer
Tuesday, September 27 <sup>th</sup>	7:00 am – 5:30 pm	Grand Foyer

### Lost & Found

Any found items should be given to ACCP Staff at the Registration Desk in the Grand Foyer. Persons wishing to retrieve a lost item should also contact ACCP Staff at the ACCP Registration Desk.



# ACCP

AMERICAN COLLEGE OF CLINICAL PHARMACOLOGY  
Advancing Clinical Care through Pharmacology®

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### American College of Clinical Pharmacology 2016 Program Committee

#### Co-chairs:

Vikram Arya, PhD  
Manish Gupta, PhD  
Honghui Zhou, PhD

#### Members:

Nageshwar R. Budha, PhD  
Lawrence J. Cohen, PharmD  
Amelia N. Deitchman, PharmD  
Nilima A. Kshirsagar, MBBS, MD, PhD  
Nitin Mehrotra, PhD  
Robert J. Noveck, MD, PhD, CPI  
Stephan Schmidt, PhD  
Jayabharathi Vaidyanathan, PhD  
John van den Anker, MD, PhD

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# Letter of Welcome from President & Program Co-chairs

## Welcome to the 2016 ACCP Annual Meeting!

### *Clinical Pharmacology: Discovery and Application in the Era of Precision Medicine*

Dear Colleague:

It is our pleasure to welcome you to the **2016 Annual Meeting** of the **American College of Clinical Pharmacology (#2016ACCP)**, ***Clinical Pharmacology: Discovery and Application in the Era of Precision Medicine***. The 2016 Annual Meeting Program Committee, co-chaired by Drs. Vikram Arya, Honghui Zhou and Manish Gupta, has developed a diverse and exceptional educational program to meet the needs of a broad spectrum of healthcare professionals and scientists with an interest in clinical pharmacology applications from research and drug development to patient care. ACCP is pleased to host a global panel of speakers from academia, industry, regulatory and clinical entities that will present programs organized into topic tracks, allowing each attendee to uniquely tailor content selection based on individual interests. Major clusters of topic areas include oncology drug development, including immuno-oncology, biologics and biosimilars, pediatric drug development and precision medicine. The exciting mix of sessions includes the use of novel and innovative clinical pharmacology tools and principles to improve drug development and therapeutics for effective treatment of disease states such as HIV/AIDS, Hepatitis C and cancer; orphan drug development; the application of the animal rule; the management of opioid dependence; the use of Big Data, physiologically-based PK/PD modeling and novel trial designs for pediatric drug development programs; improvements in clinical pharmacology labeling and streamlining of clinical pharmacology activities in early development.

New this year is a mixture of several shorter Symposia combined with our traditional four-hour format. For the first time, a select group of cutting-edge poster presentations will be hosted in an intimate setting that encourages discussion in a relaxed atmosphere. Of special note are Student & Trainee-focused programs that provide exposure to cutting-edge science and career development.

Poster Sessions held on Sunday and Monday evening will focus on new findings and preliminary data presented by a wide spectrum of attendees. Socialize and network at the catered receptions during the Poster Sessions, at twice-daily tea/coffee breaks and at the Lunch & Awards Sessions on Monday and Tuesday. At the lunch session on Tuesday, Kathy Hudson, PhD, Deputy Director for Science, Outreach and Policy at the National Institute of Health, will present the Keynote address.

Experience for yourself how ACCP makes a difference by providing healthcare professionals and scientists with a forum to exchange knowledge and ideas that promote and expand the value of clinical pharmacology in healthcare and drug development.

Please note that there is complimentary Internet access provided to all Annual Meeting attendees in guest rooms for the duration of the meeting. All of the educational sessions, social events and networking will be held at the hotel, facilitating the ease with which meeting attendees can participate in events. With the hotel's convenient location to the White Flint Metro Station, there is easy access to downtown Bethesda, Rockville, Washington, DC and northern Virginia attractions, making it easy to enjoy all that the Washington metro area has to offer.

ACCP remains an accredited provider of Continuing Medical Education (CME) and Continuing Pharmacy Education (CPE) credits for our educational events, provided to meeting attendees at no additional cost.

We welcome you to an outstanding 2016 ACCP Annual Meeting and look forward to feedback about your participation!



Bernd Meibohm, PhD  
ACCP President



Vikram Arya, PhD  
Program Co-chair



Honghui Zhou, PhD  
Program Co-chair



Manish Gupta, PhD  
Program Co-chair



# ACCP

AMERICAN COLLEGE OF CLINICAL PHARMACOLOGY  
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## Program at a Glance

*The 2016 ACCP Annual Meeting is supported in part by an Educational Grant from Pfizer Inc*

### FRIDAY, SEPTEMBER 23, 2016

**ACCP Registration Desk Open** | 4:00 – 7:00 pm  
*Grand Foyer*

**ACCP Executive Committee Meeting** | 6:00 – 9:00 pm  
*Middlebrook*

### SATURDAY, SEPTEMBER 24, 2016

**ACCP Registration Desk Open** | 7:00 am – 5:30 pm  
*Grand Foyer*

**Continental Breakfast** | 7:30 – 8:30 am  
*Glen Echo Foyer*

**ACCP Board of Regents Meeting** | 8:00 am – 1:00 pm  
*Glen Echo*

**Pre-meeting Workshop 1** | 8:00 am – 12:00 pm  
Translational Pharmacokinetics/Pharmacodynamics in  
Biotherapeutic Minimum Anticipated Biological Effect Level  
Dose Selection & Novel Protein Scaffolds  
CO-CHAIRS: Honghui Zhou, PhD and Rong Shi, PhD  
*Ballroom Salon H*

**Pre-meeting Workshop 2** | 8:00 am – 12:00 pm  
Combating HIV/AIDS: Treatment, Pharmacogenetics & Pre-  
exposure Prophylaxis  
CO-CHAIRS: Sam Hariforoosh, PharmD, PhD and Ganesh Cherala, PhD  
*Ballroom Salon G*

**Pre-meeting Workshop 3** | 1:30 – 5:30 pm  
Improving Therapeutics to Better Care for Older Adults &  
the Young  
CO-CHAIRS: S.W. Johnny Lau, PhD and Thomas Eissing, PhD  
*Ballroom Salon H*

**Pre-meeting Workshop 4** | 1:30 – 5:00 pm  
The Other Bound of the Therapeutic Window: Exposure-Safety  
Analysis to Inform Dosing Decisions in Oncology  
CO-CHAIRS: Justin C. Earp, PhD and Anshu Marathe, PhD  
*Ballroom Salon G*

**ACCP Finance Committee Meeting** | 3:00 – 5:00 pm  
*Middlebrook*

**Regents & Awards Reception (invitation only)**  
5:30 – 6:30 pm | *Brookside Foyer*

**Regents & Awards Dinner (invitation only)**  
6:30 – 8:30 pm | *Brookside A&B*

### SUNDAY, SEPTEMBER 25, 2016

**ACCP Registration Desk Open** | 6:30 am – 7:00 pm  
*Grand Foyer*

**Continental Breakfast** | 7:00 – 8:00 am  
*Grand Foyer*

**Welcome and Opening Remarks by President**  
7:45 – 8:00 am | *Ballroom Salon A-C*

**Symposium 1** | 8:00 am – 12:00 pm  
Clinical Pharmacology as a Cornerstone for Development of  
Products Under the Animal Rule: Determining an Effective Dose  
in Humans  
CO-CHAIRS: Nitin Mehrotra, PhD and Kimberly L. Bergman, PharmD  
*Ballroom Salon A-C*

**Symposium 2** | 8:00 – 9:30 am  
A 360° View of Immunogenicity: Qualitative & Quantitative  
Assessments to Understand Its Implications on  
Pharmacokinetics, Safety & Efficacy  
CO-CHAIRS: Chaitali Passey, PhD and Sumit Rawal, PhD  
*Ballroom Salon D*

**Symposium 3** | 10:00 am – 12:00 pm  
Helping Advance the Immuno-Oncology Revolution: Trends in  
Translational Immuno-Oncology  
CO-CHAIRS: Sree Kasichayanula, PhD and Yu-Nien (Tom) Sun, PhD  
*Ballroom Salon D*

**Honors & Awards Committee Meeting** | 12:00 – 1:30 pm  
*Forest Glen*

**2016 – 2017 Program Committee Meeting**  
12:00 – 1:30 pm | *Glen Echo*

**Membership Committee Meeting** | 12:00 – 1:30 pm  
*Middlebrook*

**Public Policy Committee Meeting** | 12:00 – 1:30 pm  
*Timberlawn*

**Symposium 4** | 1:30 – 5:30 pm  
Clinical Development of Biologics: Current Strategy, Challenges  
& Future Considerations  
CO-CHAIRS: Gaurav Bajaj, PhD and Ping Zhao, PhD  
*Ballroom Salon A-C*

**Symposium 5** | 1:30 – 3:00 pm  
Addressing Opioid Dependence: Now is the Time  
CO-CHAIRS: Lorraine M. Rusch, PhD and Michael J. Fossler, Jr, PharmD,  
PhD  
*Ballroom Salon D*

**Student Panel Discussion & Career Guidance**  
2:00 – 3:30 pm | *White Flint Amphitheater*

**Student Podium Presentations** | 3:30 – 4:30 pm  
*White Flint Amphitheater*

**Symposium 6** | 3:30 – 5:30 pm  
Treatment of Hepatitis C with Direct-acting Antiviral Drugs:  
Opportunities & Challenges  
CO-CHAIRS: Vikram Arya, PhD and Shirley Seo, PhD  
*Ballroom Salon D*

**Student Networking Reception** | 4:30 – 5:30 pm  
*Brookside Foyer*

**Opening Reception & Poster Session 1 & Exhibits**  
5:30 – 7:30 pm | *Ballroom Salon E-H*

**Student Poster Tour** | 5:45 – 6:30 pm  
*Meet at ACCP Registration Desk at 5:30 pm*



# Program at a Glance

## MONDAY, SEPTEMBER 26, 2016

**ACCP Registration Desk Open** | 7:00 am – 7:00 pm  
*Grand Foyer*

**Continental Breakfast** | 7:00 – 8:00 am  
*Ballroom Salon E-H*

**Exhibit Hall Open** | 7:00 – 10:00 am  
*Ballroom Salon E-H*

**Annual Business Meeting** | 7:15 – 8:00 am  
*Ballroom Salon A-C*

**Symposium 7** | 8:00 am – 12:00 pm  
**Establishing Biosimilarity: The European Perception, Experience & Future Trends**  
CO-CHAIRS: Hildegard Sourgens, MD, PhD and Hartmut Derendorf, PhD  
*Ballroom Salon A-C*

**Symposium 8** | 8:00 – 9:30 am  
**Informing Pediatric Development Programs: Leveraging Big Data**  
CO-CHAIRS: Jeffrey Barrett, PhD and Lily (Yeruk) Mulugeta, PharmD  
*Ballroom Salon D*

**Symposium 9** | 10:00 am – 12:00 pm  
**Little Children, Big Challenges: The Problems for Neonatal Drug Trials & the Way Forward**  
CO-CHAIRS: Jian Wang, PhD and John van den Anker, MD, PhD  
*Ballroom Salon D*

**Lunch Buffet** | 11:45 am – 1:45 pm | *Grand Foyer A-D*

**Lunch & Awards Session** | 12:10 – 1:20 pm  
*Ballroom Salon A-D*

- Distinguished Investigator Award
- Honorary Fellowship Award
- Nathaniel T. Kwit Memorial Distinguished Service Award
- McKeen Cattell Memorial Award
- Abstract Awards
- Member-Get-a-Member Awards

**Symposium 10** | 1:30 – 5:30 pm  
**Streamlining Clinical Pharmacology Strategies During Early Development: Assessment of Drug-Drug Interactions, Food Effect & QTc**  
CO-CHAIRS: Suraj G. Bhansali, MS, PhD and Xiao Hu, PhD  
*Ballroom Salon A-C*

**Symposium 11** | 1:30 – 3:00 pm  
**Cutting-edge Abstract Presentations**  
CO-CHAIRS: Lawrence J. Cohen, PharmD, Walter K. Kraft, MD and Amalia M. Issa, PhD  
*Ballroom Salon D*

**Exhibit Hall Open** | 3:00 – 7:30 pm | *Ballroom Salon E-H*

**Symposium 12** | 3:30 – 5:30 pm  
**Rethinking Clinical Pharmacology-related Labeling for Improved Utility & Comprehension**  
CO-CHAIRS: Joseph A. Grillo, PharmD and Julie Bullock, PharmD  
*Ballroom Salon D*

**Evening Reception & Poster Session 2 & Exhibits**  
5:30 – 7:30 pm | *Ballroom Salon E-H*

**Editorial Board Dinner (invitation only)** | 7:30 – 9:30 pm  
*Brookside A&B*

## TUESDAY, SEPTEMBER 27, 2016

**ACCP Registration Desk Open** | 7:00 am – 5:30 pm  
*Grand Foyer*

**Continental Breakfast** | 7:00 – 8:00 am  
*Ballroom Salon E-H*

**Student Event: Special Access to the Experts**  
7:00 – 8:00 am | *Ballroom Salon E-H*

**Exhibit Hall Open** | 7:00 – 10:00 am  
*Ballroom Salon E-H*

**Education Committee Meeting** | 7:00 – 8:00 am  
*Great Falls*

**Publications Committee Meeting** | 7:00 – 8:00 am  
*Middlebrook*

**Symposium 13** | 8:00 am – 12:00 pm  
**Orphan Drug Development in Adults & Pediatrics: Industry, Academia & Regulatory Perspectives**  
CO-CHAIRS: Vijay Ivaturi, PhD and Venkatesh Atul Bhattaram, PhD  
*Ballroom Salon A-C*

**Symposium 14** | 8:00 – 9:30 am  
**Clinical Applications of Physiologically-based Pharmacokinetics/ Pharmacodynamics for Pediatrics: Academic, Industry & Regulatory Perspectives**  
CO-CHAIRS: Jennifer Sheng, PhD, PharmD and Diansong Zhou, PhD  
*Ballroom Salon D*

**Symposium 15** | 10:00 – 11:45 am  
**Combination Therapy in Oncology: Challenges & Strategies in Clinical Pharmacology**  
CO-CHAIRS: Yilong Zhang, PhD and Satyendra Suryawanshi, PhD  
*Ballroom Salon D*

**Lunch Buffet** | 11:45 am – 1:45 pm | *Grand Foyer A-D*

**Lunch & Awards Session** | 12:10 – 1:20 pm  
*Ballroom Salon A-D*

- Tanabe Young Investigator Award
- Bristol-Myers Squibb Mentorship in Clinical Pharmacology Award
- Keynote Presentation: “The Precision Medicine Initiative” Kathy L. Hudson, PhD, Deputy Director for Science, Outreach and Policy at the National Inst of Health

**Symposium 16** | 1:30 – 5:30 pm  
**Clinical Pharmacology Strategies in Precision Medicine-based Drug Development & Preventive Medicine**  
CO-CHAIRS: Priyanka Jadhav, PhD, Jinshan Shen, PhD and Manoj P. Jadhav, PhD  
*Ballroom Salon A-C*

**Symposium 17** | 1:30 – 5:30 pm  
**Reproducible Visualization & Data Analysis With R**  
CHAIR: Devin Pastoor, MTOX  
*Ballroom Salon D*



# ACCP

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## Keynote Speaker



Tuesday, September 27, 2016 | 12:50 – 1:20 pm | Ballroom Salon A – D

### Kathy L. Hudson, PhD

Deputy Director for Science, Outreach and Policy at the National Inst of Health

#### **“The Precision Medicine Initiative”**

Kathy L. Hudson, PhD, is the Deputy Director for Science, Outreach and Policy at the National Inst of Health (NIH). Dr. Hudson leads the science policy, legislation, communication and outreach efforts of the NIH and serves as a senior advisor to the NIH Director. She is responsible for creating major new strategic and scientific initiatives for NIH and is currently leading the planning and creation of the President's Precision Medicine Initiative Cohort Program. Dr. Hudson was a key architect of the National Ctr for Advancing Translational Sciences and the NIH BRAIN Initiative. She directs the agency's efforts to advance biomedical research through policy development, public and stakeholder communication & education and innovative projects & partnerships.

Dr. Hudson's professional experience includes serving as the NIH Chief of Staff; Acting Deputy Director of the National Ctr for Advancing Translational Sciences, NIH; the Assistant Director of the National Human Genome Research Inst, NIH; and the founder and Director of the Genetics and Public Policy Ctr at Johns Hopkins Univ. Also at Hopkins, Dr. Hudson was an Associate Professor in the Berman Inst of Bioethics, Inst of Genetic Medicine and Dept of Pediatrics.

Dr. Hudson holds a PhD in Molecular Biology from the Univ of California at Berkeley, an MS in Microbiology from the Univ of Chicago and a BA in Biology from Carleton Coll.

## Abstract Awards Program

### **Student & Trainee Abstract Award Winners**

Student & Trainee Abstract Awards are given for the best abstracts submitted by Students & Trainees for presentation at each year's Annual Meeting.

#### **Wayne A. Colburn Memorial Award**

The Wayne A. Colburn Memorial Award honors the memory of the late Wayne A. Colburn, former ACCP President, and will be given for the best paper among the Student & Trainee Award Winners, as judged by the Program Committee during the Poster Sessions at the Annual Meeting. The winner will be announced during the Monday luncheon and the author will give a short talk outlining the findings of the study in Symposium 11.

#### **New Member Abstract Award**

The New Member Abstract Award is given for the best abstract submitted by a New Member of ACCP for presentation at the Annual Meeting. Abstracts submitted by New Members will be judged during the Poster Sessions. The winner will be announced during the Monday luncheon and the author will give a short talk outlining the findings of the study in Symposium 11.

### **2016 Student & Trainee Abstract Award Winners**

Student Abstract Award Winners will present their posters at both Poster Sessions

- **Sumit Basu, PhD (Poster #089)** *Univ of Florida, Orlando, FL*
- **Kristina M. Brooks, PharmD (Poster #041)** *National Inst of Health, Bethesda, MD*
- **Amelia N. Deitchman, PharmD (Poster #013)** *Univ of Florida, Orlando, FL*
- **Asma El-Zailik, BS (Poster #014)** *Univ of Houston, Houston, TX*
- **Edwin Lam, PharmD (Poster #061)** *Long Island Univ, Brooklyn, NY*
- **Naveen Mangal, BS, MS (Poster #088)** *Univ of Florida, Orlando, FL*
- **Mahua Sarkar, MS (Poster #001)** *Univ of Houston, Houston, TX*
- **Tanaya Vaidya, MS (Poster #040)** *Univ of Florida, Orlando, FL*
- **Jessica Wojciechowski (Poster #076)** *Univ of South Australia, Adelaide, AU*

## 2016 ACCP Recognition Award Winners



### Distinguished Investigator Award

Monday, September 26, 2016 | 12:15 – 12:35 pm | Ballroom Salon A – D

#### “From Methotrexate to Targeted Therapies for Cancer: Individualizing the Approach”

**Bruce A. Chabner, MD** – Professor of Medicine, Harvard Medical School; Emeritus Director of Clinical Research, Massachusetts General Hosp Cancer Ctr; Co-leader, Translational Pharmacology & Early Therapeutic Trials Program at the Dana Farber/ Harvard Cancer Ctr

The Distinguished Investigator Award is given annually and is intended to recognize superior scientific expertise and accomplishments by a senior investigator, usually involving a distinct area of research in basic or clinical pharmacology, for which the individual is internationally known. The

candidate need not be a Member or Fellow of ACCP.

Dr. Chabner has performed seminal and extensive work in the field of cancer drug discovery and development. His lifelong contributions to the field of clinical pharmacology and oncology make him a worthy recipient of the 2016 ACCP Distinguished Investigator Award.



### Honorary Fellowship Award

Monday, September 26, 2016 | 12:35 – 12:55 pm | Ballroom Salon A – D

#### “Optimal Design in Pharmacometrics and Dose of Favipiravir for Ebola”

**France Mentré, PhD, MD** – Director of Research, Vice Director, Graduate School of Public Health, Univ of Paris & Head, Biostatistics Dept, Bichat Hosp

The Honorary Fellowship Award is given annually to a Non-member of ACCP and is meant to recognize primary activities within the immediate domain of clinical pharmacology. The award recognizes overall contributions to the field, rather than any particular scientific work, by a senior investigator or authority having a national or international reputation in the scientific, public service, legislative, governmental or other area of endeavor impacting the field.

Dr. Mentré holds leadership positions in several scientific organizations which support clinical pharmacology research and is the current Chair of the Executive Committee of the World Conference on Pharmacometrics and an Associate Editor of the *Journal of Pharmacometrics and Systems Pharmacology*. She has made substantial contributions to the field of clinical pharmacology through research and training of basic and clinical pharmacologists, as well as through the development of tools to facilitate research in clinical pharmacology, making her a fitting recipient of the 2016 ACCP Honorary Fellowship Award.

### 2016 Honors & Awards Committee

Vera Donnenberg, PhD • Claude Abdallah, MD, MSc Pharm • April Barbour, PhD  
Steven J. Crosby, MA, BSP, RPh • Navin Goyal, PhD • Howard Greenberg, MD, MSE, MBA  
Manoj P. Jadhav, PhD • Jatinder Mukker, PhD • Eric Olson, PhD  
Laurent Vernillet, PharmD, PhD • Peter Wiernik, MD



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## 2016 ACCP Recognition Award Winners



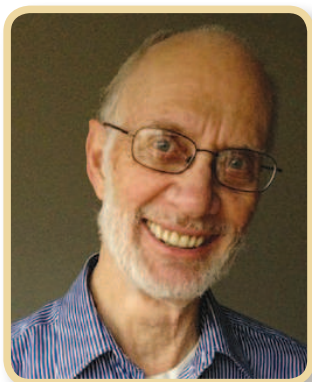
### **Nathaniel T. Kwit Memorial Distinguished Service Award**

**Monday, September 26, 2016 | 12:55 – 1:15 pm | Ballroom Salon A – D**

**Margaret Hamburg, MD – Foreign Secretary for the National Academy of Medicine; previously Commissioner, US Food & Drug Administration**

The Nathaniel T. Kwit Memorial Distinguished Service Award is given in memory of the late Nathaniel T. Kwit, MD, FCP, a founding Fellow of ACCP, who served as a Regent for 5 years and as Treasurer for 20 years. The primary intent of this award is to recognize accomplishments of a general nature which benefit the field of clinical pharmacology. These may be in the area of teaching, administration, service with ACCP or long-term and wide-ranging scientific studies having practical importance and other service-related functions. It is differentiated from the Distinguished Investigator Award in that it is not intended to recognize any distinct area of scientific investigation, but rather an overall contribution to the field. The candidate need not be an ACCP Member or Fellow.

While Commissioner of the US Food & Drug Administration, Dr. Hamburg supported regulatory initiatives for personalized drug therapy. Her extensive contributions, directly and indirectly relevant to clinical pharmacology, make her an outstanding recipient of the 2016 Nathaniel T. Kwit Memorial Distinguished Service Award.



### **McKeen Cattell Memorial Award**

**Monday, September 26, 2016 | 1:15 – 1:20 pm | Ballroom Salon A – D**

**Daniel A. Spyker, PhD, MD – Consulting Senior Director, Drug Safety & Pharmacovigilance, Alexza Pharmaceuticals Inc; Adjunct Professor, Dept of Internal Medicine, Uniformed Services Univ of Health Sciences; Adjunct Assistant Professor of Emergency Medicine, Oregon Health & Science Univ**

The McKeen Cattell Memorial Award is made in memory of the late McKeen Cattell, MD, PhD, FCP, the first editor of *The Journal of Clinical Pharmacology* (JCP) and co-founder of ACCP. This award is made annually, recognizing an outstanding research paper published in the JCP during the preceding year. The award is typically presented to the first author of the paper.

This year's award-winning journal article is: **"Multiple-dose Pharmacokinetics of Inhaled Loxapine in Subjects on Chronic, Stable Antipsychotic Regimens"** Authors: Daniel A. Spyker,

PhD, MD, Robert A. Riesenber, MD and James V. Cassella, PhD. Published in *The Journal of Clinical Pharmacology* Volume 55, Issue 9, pages 985–994, September 2015.



## 2016 ACCP Recognition Award Winners



### **Tanabe Young Investigator Award**

Tuesday, September 27, 2016 | 12:15 – 12:30 pm | Ballroom Salon A – D

#### **“How Quantitative & Systems Pharmacology Can Bring Value to R&D and, Ultimately, to the Patient”**

**Stephan Schmidt, PhD – Assistant Professor, Univ of Florida, Ctr for Pharmacometrics & Systems Pharmacology; Chair of the Int’l Pharmaceutical Federation’s Special Interest Group on Pharmacometrics & Systems Pharmacology; Incoming Chair of the ASCPT’s Special Interest Group on Systems Pharmacology**

The Tanabe Young Investigator Award recognizes the significant contributions of an investigator who has made unusual strides in research related to clinical pharmacology and whose career shows promise of outstanding achievements at a relatively early stage, typically 10 – 12 years post-research degree. The candidate need not be a Member or Fellow of ACCP.

Dr. Schmidt’s research focuses on the application of quantitative systems pharmacology to address clinically-relevant questions in the areas of antimicrobial chemotherapy, pediatrics, diabetes, cardiovascular safety and post-menopausal osteoporosis. He is a bright young investigator in drug modeling and clinical pharmacology and has an extraordinary track record of achievement since joining the faculty at the Univ of Florida in 2012, making him a deserving recipient of the 2016 Tanabe Young Investigator Award.



### **Bristol-Myers Squibb Mentorship in Clinical Pharmacology Award**

Tuesday, September 27, 2016 | 12:30 – 12:50 pm | Ballroom Salon A – D

#### **“From Books to Grooks”**

**Richard Brundage, PharmD, PhD – Professor of Experimental & Clinical Pharmacology, Univ of Minnesota, Coll of Pharmacy**

The Bristol-Myers Squibb Mentorship in Clinical Pharmacology Award is given annually to an awardee who demonstrates exemplary promotion of clinical pharmacology, with emphasis on training/guidance of junior scientists and/or colleagues.

Dr. Brundage is widely recognized for exceptional mentoring abilities and unfailing dedication to students and trainees. He is an extraordinary teacher and an enthusiastic mentor who is passionate about his work. His academic accomplishments and mentorship of the current and future generation of clinical pharmacologists and pharmacists make Dr. Brundage a well-deserving recipient of the 2016 Bristol-Myers Squibb Mentorship in Clinical Pharmacology Award.



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## Educational Accreditation

### Accreditation Statements



The American College of Clinical Pharmacology is accredited by the Accreditation Council for Pharmacy Education (ACPE) as a provider of continuing pharmacy education.

The ACPE universal program numbers assigned and hours of credit are noted within each segment of the program for a maximum of 28 Contact Hours. All CPE activities are application-based.



The American College of Clinical Pharmacology is accredited by the Accreditation Council for Continuing Medical Education (ACCME) to provide continuing medical education for physicians.

### Designation Statement

The American College of Clinical Pharmacology designates this live educational activity for a maximum of 28 *AMA PRA Category 1 Credits™*. Physicians should claim only the credit commensurate with the extent of their participation in the activity.

Symposium 7: Establishing Biosimilarity: The European Perception, Experience & Future Trends has been planned and implemented in accordance with the accreditation requirements and policies of the Accreditation Council for Continuing Medical Education (ACCME) through the joint providership of the American College of Clinical Pharmacology and the European Federation for Exploratory Medicines Development. The American College of Clinical Pharmacology is accredited by the ACCME to provide continuing medical education for physicians.

### Continuing Education Process for 2016

Attendees interested in earning continuing education credit should specifically request that when they register for the 2016 Annual Meeting. Attendees who indicated they want to obtain continuing education credit will be provided with access to post-event tests related to the courses they attend. Completion of the post-event tests is required to earn the credit and to print continuing education credit certificates. Post-event tests require a 75% passing score.

Attendees seeking CPE credit should, if they have not already done so, provide ACCP with their NABP Profile Number and the month and date of their birthday via email at [CE@ACCP1.org](mailto:CE@ACCP1.org). The profile number and birthday information is used when ACCP sends CPE credit information to the National Association of Boards of Pharmacy (NABP) using CPE Monitor. Pharmacists/pharmacy technicians are asked to obtain their NABP e-Profile ID by contacting the National Association of Boards of Pharmacy or by contacting NABP Customer Service at 847-391-4406.

Please note: If pharmacists/pharmacy technicians fail to set up their NABP e-Profile Identification Number, ACCP will not be able to provide the ACPE/NABP with the information which will allow pharmacists/pharmacy technicians to track completed continuing pharmacy education credit(s). ACCP cannot be responsible for individuals who have not taken the necessary steps to obtain their NABP e-Profile Identification Number and who have not provided this to ACCP prior to CPE post-event testing. For more information, or for answers to Frequently Asked Questions regarding CPE Monitor, please visit Accreditation Council for Pharmacy Education.

### What is CPE Monitor?



CPE Monitor is a national, collaborative effort by ACPE and the National Association of Boards of Pharmacy (NABP) to provide an electronic system for pharmacists/pharmacy technicians to track their completed Continuing Pharmacy Education (CPE) credits. It also offers state boards of pharmacy the opportunity to electronically authenticate the CPE units completed by their licensees, rather than requiring pharmacists/pharmacy technicians to submit proof of completion statements upon request or for random audits.



## Educational Activities

*Spend an hour each month with ACCP  
to stay in the forefront of your field!*

### ACCP offers several ways to spend that hour each month!

Stay current and earn continuing education credit!

- **The Journal of Clinical Pharmacology CE Program:** Each month an outstanding, relevant article is selected to offer CE credits; available On Demand;
- **ACCP Virtual Journal Club:** Offered at least eight times per year, this is an opportunity to interact with the author of a selected article in a webinar format, including an engaging Q&A session; available as a live webinar, then On Demand;
- **ACCP Fundamentals Tutorials:** Designed to provide a “not too technical” overview of clinical pharmacology, this series is available On Demand;
- **Therapeutic Dilemmas:** This webinar series will continue in the fall of 2016 and is available as a live webinar, then On Demand;
- **On Demand Library:** The ACCP CE Catalog now contains over 20 sessions that you can view On Demand – any time, any place!




**Virtual Journal Club**

### Couldn't attend all the sessions or have a colleague that couldn't attend?

Coming in October – the following sessions from the 2016 ACCP Annual Meeting will be available On Demand:

Attendees of the 2016 Annual Meeting can access these events at no charge.

- **Symposium 1:** *Clinical Pharmacology as a Cornerstone for Development of Products Under the Animal Rule: Determining an Effective Dose in Humans*
- **Symposium 2:** A 360° View of Immunogenicity: Qualitative & Quantitative Assessments to Understand Its Implications on Pharmacokinetics, Safety & Efficacy
- **Symposium 3:** Helping Advance the Immuno-Oncology Revolution: Trends in Translational Immuno-Oncology
- **Symposium 4:** Clinical Development of Biologics: Current Strategy, Challenges & Future Considerations
- **Symposium 5:** Addressing Opioid Dependence: Now is the Time
- **Symposium 6:** Treatment of Hepatitis C with Direct-acting Antiviral Drugs: Opportunities & Challenges



To view the entire  
ACCP CE Catalog, visit  
<https://ce.accp1.org/>



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## Faculty Disclosure Information

The following Faculty participants have indicated they have a disclosure related to the content of their presentation.

**Gaurav Bajaj:** employee (salary) – Bristol-Myers Squibb Co

**Jeffrey Barrett:** employee (salary) – Sanofi

**Jonathan Benjamin:** employee (salary/stock) – Amgen Inc

**Suraj G. Bhansali:** employee (salary/stock) – Novartis Pharmaceuticals Corp

**Indranil Bhattacharya:** employee (salary/ownership interest) – Pfizer Inc; former employee (ownership interest) – GlaxoSmithKline plc

**\*Satjit Brar:** employee (salary/stock options) – Pfizer Inc

**Julie Bullock:** employee (salary) – d3 Medicine LLC

**\*Ganesh Cherala:** employee (salary) – Novo Nordisk Inc

**\*Andrew T. Chow:** employee (salary/stock) – Amgen Inc

**\*James Cloyd III:** licensing agreement (royalties) – Univ of Minnesota, Ligand Pharmaceuticals Inc; licensing agreement (future royalties) – Univ of Minnesota, Allaysis LLC; advisory board and licensing agreement (honorarium/royalty agreement) – Lundbeck; consulting (fees) – Upsher-Smith Laboratories Inc, Xeris Pharmaceuticals Inc, Neurelis Inc, UCB Inc, Eisai Co Ltd

**Dora W. Cohen:** employee (salary) – Amgen Inc

**\*Lawrence J. Cohen:** consultant and committee member (consulting fee) – PharMerica

**\*Alfred Corey:** former employee (salary) – PAREXEL Intl; former employee (stock) – GlaxoSmithKline plc

**\*Gabriele Dallmann:** consulting (fees) – various pharma companies

**John D. Davis:** employee (salary/stock options) – Regeneron Pharmaceuticals Inc

**\*Paul Declerck:** speaking and teaching, non-product related (honoraria) – Celltrion Inc, Hospira, Novo Nordisk Inc, Pfizer Inc, Roche

**\*Hartmut Derendorf:** consulting (honorarium) – PK PDyne Inc

**Thomas Eissing:** employee (salary/stock) – Bayer Technology Svcs Co/ Bayer AG

**Raffaella Faggioni:** employee (salary/stock) – MedImmune LLC/ AstraZeneca plc

**Michael J. Fossler, Jr:** employee (salary/stock) – Trevena Inc

**Leonid Gibiansky:** consulting (fees) – multiple pharmaceutical companies; published papers in collaboration with Genentech Inc, Bristol-Myers Squibb Co and Roche during 2015

**\*Varun Goel:** employee (salary/stock) – Novartis Inst for Biomedical Research; spouse: employee (salary/stock) – Alnylam Pharmaceuticals, Vertex Pharmaceuticals Inc

**Adam Golden:** consulting (fees) – Magellan Health Inc

**George R. Gunn, III:** employee (salary/stock/stock options) – Janssen Research & Development LLC; patent licensing (payment) – Advaxis Inc

**Manish Gupta:** employee (salary/stock) – Bristol-Myers Squibb Co

**R. Donald Harvey:** clinical trial conduct (research funding to Emory) – Amgen Inc, ArQule Inc, AstraZeneca plc, Aveo Pharmaceuticals Inc, Bristol-Myers Squibb Co, Celgene Corp, Cleave Biosciences, Calithera Biosciences Inc, Eli Lilly & Co, Merck & Co, Novartis, Pfizer Inc, Sanofi, Takeda Pharmaceuticals USA Inc; advisory board (honorarium) – Bristol-Myers Squibb Co, Takeda Pharmaceuticals USA Inc

**Tycho Heimbach:** employee (salary/stock options) – Novartis Pharmaceuticals Corp

**Bart Hendriks:** employee (salary/stock options) – Merrimack Pharmaceuticals Inc

**\*Craig W. Hendrix:** consulting (fees) – Oak Crest Inst of Science, Univ of California, Los Angeles, Univ of Washington; principal investigator (pending research contract) – Viiv Healthcare, GlaxoSmithKline plc through Johns Hopkins

**Xiao Hu:** employee (salary/bonus/stock) – Biogen Inc

**Kaori Ito:** employee (salary/stock) – Pfizer Inc

**Manoj P. Jadhav:** employee (salary) – CRC Pharma LLC

**Priyanka Jadhav:** employee (salary) – CRC Pharma LLC

**Xiling Jiang:** employee (salary/stock) – Johnson & Johnson

**Trevor N. Johnson:** employee (salary) – Simcyp Ltd (part of Certara)

**\*Mats O. Karlsson:** consulting (fees) – Boehringer Ingelheim GmbH, Pfizer Inc; (stock) – Pharmetheus AB, Wellhagen & Karlsson AB

**Sree Kasichayanula:** employee (salary/stock) – Amgen Inc

**\*Parag Kumar:** collaboration (product) – Matinas BioPharma Holdings Inc

**\*J. Steven Leeder:** consulting (fees go to employer – US Food & Drug Administration) – Neurocrine Biosciences Inc

**Lawrence J. Lesko:** consulting (fees) – Amgen Inc, Biogen Inc, Relypsy Inc, Merrimack Pharmaceuticals Inc

**Chunze Li:** employee (salary/stock) – Genentech Inc

**Donald E. Mager:** Principal Investigator (potential research grant for systems modeling in immuno-oncology) – Bristol-Myers Squibb Co

**Christina L. Mayer:** employee (salary/stock) – Janssen Research & Development LLC

**Bernd Meibohm:** consulting (fees) – Alexion Pharmaceuticals Inc, AstraZeneca plc, Boehringer Ingelheim GmbH, Biogen Inc, F Hoffmann-La Roche AG, Merck KGaA, Novartis, Sanofi, Teva Pharmaceutical Industries Ltd, Tonix Pharmaceuticals Holding Corp

**Prasun Mishra:** employee (salary/stock) – Genentech Inc – a subsidiary of Roche group; former employee (salary/stock) – Gilead Sciences Inc

# Faculty Disclosure Information

**Diane R. Mould:** consulting (salary) – Projections Research Inc

**Cara Nelson:** employee (salary) – Gilead Sciences Inc

**Chaitali Passey:** employee (salary) – Bristol-Myers Squibb Co; former employee (salary) – Merck & Co

**\*Ronald J. Portman:** employee (salary/stock) – Novartis Pharmaceuticals Corp

**Sumit Rawal:** employee (salary) – Regeneron Pharmaceuticals Inc

**\*Zachary Rome:** employee (salary) – Patagonia Pharmaceuticals LLC

**Karen Rowland Yeo:** employee (salary) – Simcyp Ltd (Certara); spouse: employee (salary) – Pfizer Inc

**Lorraine M. Rusch:** employee (salary) Celerion Inc; spouse: former employee (stock) – Cara Therapeutics Inc, Acorda Therapeutics Inc

**\*Huub Schellekens:** member ad board (honorarium) – Merck Serono & Merck & Co; consulting (fees) – Eagle Pharmaceuticals Inc

**Jan-Frederik Schlender:** employee (salary) – Bayer Technology Svcs GmbH

**Edward M. Sellers:** consulting (fees) – DL Global Partners Inc

**Jinshan Shen:** employee (salary/stock options) – Radius Health Inc; former employee (salary/stock options/restricted stock) – Vertex Pharmaceuticals Inc

**Jennifer Sheng:** employee (salary) – Bristol-Myers Squibb Co

**Rong Shi:** employee (salary/stock) – Bristol-Myers Squibb Co

**Vikram Sinha:** employee (salary) – Merck & Co

**\*Konstantine W. Skordos:** employee (salary/stock) – Novartis Inst for Biomedical Research; former employee (salary/stock) – Biogen Inc

**\*P. Brian Smith:** consulting (fees) – Abbvie Inc

**\*Hildegard Sourgens:** consulting, medical writing (honoraria) – Bayer AG, Formycon AG, mibe GmbH Arzneimittel, Max Zeller Söhne AG

**Sven Stegemann:** employee part-time (salary) – Capsugel

**\*Mark Sulkowski:** consultant/advisory board (honorarium) – AbbVie Inc, Bristol-Myers Squibb Co, Cocrystal Pharma Inc, Gilead Sciences Inc, Merck & Co, Janssen Pharmaceuticals Inc, Trek Therapeutics PBC

**Yu-Nien (Tom) Sun:** employee (salary/stock) – Johnson & Johnson

**Satyendra Suryawanshi:** employee (salary/stock) – Bristol-Myers Squibb Co

**Zheng Yang:** employee (salary/stock/stock options) – Bristol-Myers Squibb Co

**Yilong Zhang:** employee (salary/stock) – Amgen Inc

**Diansong Zhou:** employee (salary) – AstraZeneca plc

**Honghui Zhou:** employee (salary/stock/stock options) – Johnson & Johnson

## The following Faculty have indicated they have no disclosures related to their presentation:

**Darrell R. Abernethy**

**\*Bilal S. AbuAsal**

**\*Hae-Young Ahn**

**\*Shashi Amur**

**\*Vikram Arya**

**\*Gerri Baer**

**\*Kimberly L. Bergman**

**\*Venkatesh Atul Bhattaram**

**Eric Brodsky**

**\*Gilbert J. Burckart**

**Leonard Campanello**

**Ruth S. Day**

**\*Gustavo F. Doncel**

**Sir Gordon W. Duff**

**\*Justin C. Earp**

**Jeffrey Florian**

**Christine Garnett**

**Jogaroo V. Gobburu**

**Joseph A. Grillo**

**\*Sam Hariforoosh**

**\*Amalia M. Issa**

**\*Vijay Ivaturi**

**Brian Jacobs**

**\*Devanand Jillapalli**

**\*Shyam Kottilil**

**\*Walter K. Kraft**

**S.W. Johnny Lau**

**Jinhee Lee**

**Jiang Liu**

**Qi Liu**

**\*Lian Ma**

**\*Anshu Marathe**

**\*Susan McCune**

**\*Nitin Mehrotra**

**\*Jonathan P. Moorman**

**Lily (Yeruk) Mulugeta**

**Michael Pacanowski**

**Devin Pastoor**

**\*Andrea M. Powell**

**Mattia Proserpi**

**\*Atiqur Rahman**

**Amy Rosenberg**

**\*Anindya Roy**

**\*Shirley Seo**

**Patricia W. Slattum**

**\*John van den Anker**

**\*Jian Wang**

**Yow-Ming C. Wang**

**\*Lynne Yao**

**Anne Zajicek**

**Hong Zhao**

**Ping Zhao**

\*This disclosure list includes all 2016 Annual Meeting Faculty. Continuing education credit is offered for ten of the 17 available Workshops and Symposia. The Faculty participating in Workshops and Symposia offering CE credit are noted with an asterisk.

## The following activity planners have indicated they have disclosures:

**Nageshwar Budha:** employee (salary) – Genentech Inc; former employee (stock) – Hoffmann-La Roche

**Lawrence J. Cohen:** consultant and committee member (consulting fee) – PharMerica

**Manish Gupta:** employee (salary/stock) – Bristol-Myers Squibb Co

**Honghui Zhou:** employee (salary/stock/stock options) – Johnson & Johnson

## The following planners have indicated they have no disclosures:

**Vikram Arya**

**Amelia N. Deitchman**

**Nilima A. Kshirsagar**

**Nitin Mehrotra**

**Robert Noveck**

**Stephan Schmidt**

**Jayabharathi Vaidyanathan**

**John van den Anker**



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## Pre-meeting Workshops

**SATURDAY, SEPTEMBER 24, 2016 | Pre-meeting Workshop 1 | 8:00 am – 12:00 pm**

### **BALLROOM SALON H**

## *Translational Pharmacokinetics/ Pharmacodynamics in Biotherapeutic Minimum Anticipated Biological Effect Level Dose Selection & Novel Protein Scaffolds*

### **DISCOVERY TRACK**

#### **CO-CHAIRS:**

**Honghui Zhou, PhD**, Senior Director & Janssen Fellow, Janssen Research & Development LLC

**Rong Shi, PhD**, Clinical Pharmacology Lead, Bristol-Myers Squibb Co

#### **TARGET AUDIENCE:**

The target audience includes preclinical and translational pharmacokinetic and pharmacodynamic (PK/PD) scientists, drug development scientists, clinical pharmacologists and those working in clinical and regulatory settings.

#### **GOALS AND OBJECTIVES:**

Following completion of this activity, the learner will be able to:

1. Discuss the advantages of novel scaffolds (eg, modified ADCC/CDC response, tissue penetration, specific targeting) & potential challenges (eg, PK, immunogenicity) relative to traditional monoclonal antibodies;
2. Discuss approaches to address these challenges and to maximize the advantages of using translational and PK/PD tools;
3. Have a holistic understanding of the available translational and PK/PD tools to face the challenges of drug development with novel scaffolds;
4. Provide an overview and share knowledge on lessons learned about the TeGenero anti-CD28 antagonist TNG1412 cytokine storm incidence;
5. Provide a comprehensive understanding of the concept of the Minimum Anticipated Biological Effect Level (MABEL) by Sir Gordon Duff;
6. Demonstrate examples of MABEL calculations in biologics programs;
7. Discuss the challenges and methodologies in calculating MABEL for First-in-Human starting dose;
8. Discuss under what circumstances the MABEL approach for First-in-Human starting dose should be used.

**8:00 – 8:10 am**

#### **Introduction**

*Honghui Zhou, PhD, Senior Director & Janssen Fellow, Janssen Research & Development LLC and Rong Shi, PhD, Clinical Pharmacology Lead, Bristol-Myers Squibb Co*

**8:10 – 8:40 am**

### **16 Translational Considerations in Developing**

#### **Bispecific Antibodies: What Can We Learn from Mechanistic PK/PD Modeling?**

*Bernd Meibohm, PhD, Professor & Associate Dean, Univ of Tennessee Health Science Ctr, Coll of Pharmacy*

**8:40 – 9:10 am**

#### **Experience in Oncology Clinical Pharmacology & Oversight on ADC: Challenges & Opportunities for Translational PK/PD in ADC Development**

*Chunze Li, PhD, Senior Scientist, Genentech Inc*

**9:10 – 9:40 am**

#### **Perspectives on Clinical Development of Abbreviated Antibody Constructs**

*Indranil Bhattacharya, PhD, Director, Pfizer Inc*

**9:40 – 10:00 am / Break**

**10:00 – 10:30 am**

#### **Intensive Review of “Expert Group on Phase 1 Clinical Trials: Final Report”**

*Sir Gordon W. Duff, Professor & Chair of the Biotechnology & Biological Sciences Research Council, St Hilda's Coll, Univ of Oxford*

**10:30 – 10:50 am**

#### **To MABEL or Not to MABEL: A Biomarker & Model-based Approach to Dose Selection for First-in-Human Studies of Biologics**

*Raffaella Faggioni, PhD, Senior Director, Clinical Pharmacology & DMPK, MedImmune LLC/AstraZeneca plc*

**10:50 – 11:10 am**

#### **PK/PD Integration of Nonclinical Data for the Determination of MABEL & First-in-Human Starting Dose: Case Studies with Biologics in Immunoscience**

*Zheng Yang, PhD, Director, Bristol-Myers Squibb Co*

**11:10 – 11:30 am**


#### **Regulatory Perspectives in Developing Biotherapeutics with Novel Protein Scaffolds**

*Yow-Ming C. Wang, PhD, Clinical Pharmacology (Biologics) Team Leader, US Food & Drug Administration*

**11:30 am – 12:00 pm**

#### **Panel Discussion**

**MED001738**



# Pre-meeting Workshops

**SATURDAY, SEPTEMBER 24, 2016 | Pre-meeting Workshop 2 | 8:00 am – 12:00 pm**

## **BALLROOM SALON G**

### *Combating HIV/AIDS: Treatment, Pharmacogenetics & Pre-exposure Prophylaxis*

#### **APPLICATION TRACK**

**Offers both CME and CPE Credit**

**UAN #0238-0000-16-002-L02-P**

**ACPE – 3.5 CONTACT HOURS/APPLICATION-BASED**

#### **CO-CHAIRS:**

**Sam Harirforoosh, PharmD, PhD**, Associate Professor, Dept of Pharmaceutical Sciences, East Tennessee State Univ, Gatton Coll of Pharmacy

**Ganesh Cherala, PhD**, Research Scientist, Research Technologies, Novo Nordisk Inc

#### **TARGET AUDIENCE:**

A better understanding of critical contributors of successful pharmacotherapy is an important step in delivering optimal healthcare. This Workshop will distill information, both evidence-based and theoretical, to the target audience of clinicians, pharmacists and scientists in practice, as well as in clinical research and drug development environments.

#### **GOALS AND OBJECTIVES:**

Following completion of this activity, the learner will be able to:

1. Describe HIV pharmacotherapy and analyze the potential of pre-exposure prophylaxis (PrEP) in combating the HIV epidemic globally;
2. Describe the influence of pharmacogenetics herein and the utility of pharmacogenetic biomarkers;
3. Demonstrate the utility of multi-purpose technologies to improve reproductive and sexual health;
4. Provide an update on drug-drug, drug-food and drug-herb interactions in HIV pharmacotherapy.

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**8:00 – 8:05 am**

#### **Introduction**

*Ganesh Cherala, PhD, Research Scientist, Research Technologies, Novo Nordisk Inc*

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**8:05 – 8:35 am**

#### **Challenges of HIV Infection in 2016**

*Jonathan P. Moorman, MD, PhD, Professor, East Tennessee State Univ, Quillen Coll of Medicine*

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**8:35 – 9:00 am**

#### **Pharmacogenetics of HIV Drugs**

*Sam Harirforoosh, PharmD, PhD, Associate Professor, Dept of Pharmaceutical Sciences, East Tennessee State Univ, Gatton Coll of Pharmacy*

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**9:00 – 9:30 am**

#### **Biomarkers in HIV & HCV Drug Development**

*Shashi Amur, PhD, Scientific Lead, Biomarker Qualification Program, US Food & Drug Administration*

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**9:30 – 10:00 am / Break**

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**10:00 – 10:30 am**

#### **HIV Pre-exposure Prophylaxis Drug Development: A Clinical Pharmacologist's Inside View**

*Craig W. Hendrix, MD, Wellcome Professor & Director, Johns Hopkins Univ School of Medicine*

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**10:30 – 11:00 am**

#### **Development of Multipurpose Technologies for the Prevention of HIV & Unintended Pregnancies: Can We Kill Two Birds with One Stone?**

*Gustavo F. Doncel, MD, PhD, Scientific Director, CONRAD & Professor of Obstetrics & Gynecology, Eastern Virginia Medical School*

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**11:00 – 11:30 am**

#### **Update on Drug Interactions in HIV**

*Parag Kumar, PharmD, Director, Clinical Pharmacokinetics Research Lab, National Inst of Health*

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**11:30 am – 12:00 pm**

#### **Panel Discussion**



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## Pre-meeting Workshops

**SATURDAY, SEPTEMBER 24, 2016 | Pre-meeting Workshop 3 | 1:30 – 5:30 pm**

### **BALLROOM SALON H**

## *Improving Therapeutics to Better Care for Older Adults & the Young*

### **DISCOVERY & APPLICATION TRACKS**

#### **CO-CHAIRS:**

**S.W. Johnny Lau, PhD**, Senior Clinical Pharmacologist, US Food & Drug Administration

**Thomas Eissing, PhD**, Head of Systems Pharmacology CV, Bayer Technology Svcs GmbH

#### **TARGET AUDIENCE:**

The target audience includes clinical pharmacologists, pharmacometricians, systems pharmacologists, pharmaceutical scientists and clinicians, as well as fellows and students from industry, academia and regulatory institutions.

#### **GOALS AND OBJECTIVES:**

Following completion of this activity, the learner will be able to:

1. Understand the issues of developing pharmacotherapy for older adults and the young;
2. Understand the regulatory aspects of developing pharmacotherapy for older adults and the young;
3. Learn from the regulatory aspect of developing drug products for the young and apply that to the older adult population;
4. Develop patient-centric or age-appropriate pharmaceutical products;
5. Apply pharmacokinetics and pharmacodynamics, as well as pharmacometrics and systems pharmacology, to better care for older adults and the young.

1:30 – 1:40 pm

#### **Introduction**

*S.W. Johnny Lau, PhD, Senior Clinical Pharmacologist, US Food & Drug Administration*

1:40 – 2:10 pm

#### **Medication Issues & Potential Solutions for Frail Older Adults**

*Adam Golden, MD, MBA, Professor, Internal Medicine, Univ of Central Florida, Coll of Medicine, Associate Chief of Staff, Geriatrics & Extended Care, Orlando Veterans Affairs Medical Ctr*

2:10 – 2:35 pm

#### **Are There Unique Issues for the Development of Drug Products for the Older Adult?**

*Darrell R. Abernethy, MD, PhD, Associate Director for Drug Safety, Office of Clinical Pharmacology, US Food & Drug Administration*

2:35 – 3:00 pm

#### **Regulatory & Clinical Pharmacology Considerations for Developing Drug Products for Pediatric Patients**

*Gilbert J. Burckart, PharmD, Associate Director for Pediatrics, US Food & Drug Administration*

3:00 – 3:30 pm / Break

3:30 – 4:00 pm

#### **Pharmaceutical Drug Product Design in the Context of Effectiveness & Safety & Their Importance in Achieving Therapeutic Outcomes**

*Sven Stegemann, PhD, Professor, Graz Univ of Technology*

4:00 – 4:30 pm

#### **Applying Pharmacokinetic & Pharmacodynamic Principles to Improve Care for Older Adults**

*Patricia W. Slattum, PharmD, PhD, Professor of Pharmacotherapy & Outcomes Science, Virginia Commonwealth Univ*


4:30 – 5:00 pm

#### **Pharmacometric Approaches to Better Care for Older Adults & the Young**

*Thomas Eissing, PhD, Head of Systems Pharmacology CV, Bayer Technology Svcs GmbH and Jan-Frederik Schlender, MSc, Pharmacist, Scientist Systems Pharmacology, Bayer Technology Svcs GmbH*

5:00 – 5:30 pm

#### **Panel Discussion**



# Pre-meeting Workshops

**SATURDAY, SEPTEMBER 24, 2016 | Pre-meeting Workshop 4 | 1:30 – 5:00 pm**

## **BALLROOM SALON G**

### *The Other Bound of the Therapeutic Window: Exposure-Safety Analysis to Inform Dosing Decisions in Oncology*

#### **DISCOVERY TRACK**

*Offers both CME and CPE Credit*

**UAN #0238-0000-16-003-L05-P**

**ACPE – 3 CONTACT HOURS/APPLICATION-BASED**

#### **CO-CHAIRS:**

**Justin C. Earp, PhD**, Pharmacometrics Reviewer, US Food & Drug Administration

**Anshu Marathe, PhD**, Team Leader, Div of Clinical Pharmacology II, US Food & Drug Administration

#### **TARGET AUDIENCE:**

The target audience includes drug development scientists from the pharmaceutical industry working in the area of oncology, academic organizations, scientists from cancer hospitals involved in drug development of oncology agents and regulatory scientists working in the area of oncology. Although the focus of the activity is in the oncology therapeutic area, the principles discussed in this topic can be applied to other therapeutic areas as well.

#### **GOALS AND OBJECTIVES:**

Following completion of this activity, the learner will be able to:

1. Understand the uniqueness of characterizing exposure-response for safety in oncology drug development programs;
2. Review current practices and identify the methodological challenges involved in conducting exposure-safety analyses for oncology agents;
3. Highlight case studies demonstrating the common methodologies/challenges in conducting oncology exposure-safety analyses;
4. Analyze and compare possible approaches for adequate exposure-safety analyses that can contribute to informed dosing decisions.

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1:30 – 1:40 pm

#### **Introduction**

*Justin C. Earp, PhD, Pharmacometrics Reviewer, US Food & Drug Administration*

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1:40 – 2:10 pm

#### **Exposure-Safety Analyses to Drive Decision Making in Oncology**

*Anshu Marathe, PhD, Team Leader, Div of Clinical Pharmacology II, US Food & Drug Administration*

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2:10 – 2:40 pm

#### **Strategies for Exposure-Safety Modeling & Simulation in Oncology with a Focus on Trial Execution Aspects**

*Mats O. Karlsson, PhD, Professor, Dept of Pharmaceutical Biosciences, Uppsala Univ*

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2:40 – 3:00 pm

#### **Q&A**

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3:00 – 3:30 pm / Break

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3:30 – 4:00 pm

#### **Exposure-Safety Analysis for Oncology Drugs: An Industry Perspective**

*Varun Goel, PhD, Fellow, Clinical Pharmacology, Novartis Inst for Biomedical Research*

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4:00 – 4:30 pm

#### **Balancing Exposure-Safety & Efficacy Analysis for Deriving Dosing in Oncology: Case Examples**

*Satjit Brar, PharmD, PhD, Associate Director, Clinical Pharmacology, Pfizer Inc*

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4:30 – 5:00 pm

#### **Panel Discussion**

*(including Konstantine W. Skordos, PhD [Novartis Inst for Biomedical Research] and Atiqur Rahman, PhD [US Food & Drug Administration])*



# ACCP

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## Symposia

**SUNDAY, SEPTEMBER 25, 2016 | Symposium 1 | 8:00 am – 12:00 pm**

### **BALLROOM SALON A-C**

## *Clinical Pharmacology as a Cornerstone for Development of Products Under the Animal Rule: Determining an Effective Dose in Humans*

### **DISCOVERY TRACK**

*Offers both CME and CPE Credit*

**UAN #0238-0000-16-004-L01-P**

**ACPE – 3.5 CONTACT HOURS/APPLICATION-BASED**

### **CO-CHAIRS:**

**Nitin Mehrotra, PhD**, Team Leader, Div of Pharmacometrics, US Food & Drug Administration

**Kimberly L. Bergman, PharmD**, Lead Pharmacologist, US Food & Drug Administration

### **TARGET AUDIENCE:**

The target audience includes clinical pharmacologists, pharmacometricians and translational medicine scientists from the pharmaceutical industry, academia and regulatory agencies who have an interest in applying and/or currently apply the principles of clinical pharmacology modeling and simulation in drug development of products under the Animal Rule.

### **GOALS AND OBJECTIVES:**

Following completion of this activity, the learner will be able to:

1. Understand the drug development and approval process of products under the Animal Rule;
2. Analyze and understand the role of clinical pharmacology modeling and simulation in dose selection in humans for development of products under the Animal Rule;
3. Highlight the case studies where clinical pharmacology modeling and simulation played a significant role in drug development or regulatory decisions.

**8:00 – 8:15 am**

### **Introduction: Setting the Stage**

*Nitin Mehrotra, PhD, Team Leader, Div of Pharmacometrics, US Food & Drug Administration*

**8:15 – 8:40 am**

### **The Animal Rule: Approval of New Drugs & Biological Products When Human Efficacy Studies Are Not Ethical or Feasible**

*Andrea M. Powell, PhD, Pharmacologist, Counter-terrorism & Emergency Coordination, US Food & Drug Administration*

**8:40 – 9:05 am**

### **The Animal Rule: The Role of Clinical Pharmacology in Determining an Effective Dose in Humans**

*Kimberly L. Bergman, PharmD, Lead Pharmacologist, US Food & Drug Administration*

**9:05 – 9:30 am**

### **Perspective on the Clinical Pharmacology Approach for Rational Choice of Drug & Dose in Product Development Under the Animal Rule: Example for Treating Patients Acutely Exposed to Myelosuppressive Doses of Radiation**

*Andrew T. Chow, PhD, Executive Director, Amgen Inc*

**9:30 – 10:00 am / Break**

**10:00 – 10:25 am**

### **The Use of Modeling & Simulation in the Raxibacumab Development Program**

*Alfred Corey, BS, Consultant, AC Pharmaco LLC*

**10:25 – 10:50 am**

### **Application of Quantitative Clinical Pharmacology in Dose Selection for Products Developed Under the Animal Rule: Case Studies**

*Lian Ma, PhD, Pharmacometrics Reviewer, US Food & Drug Administration*

**10:50 am – 12:00 pm**

### **Panel Discussion**



# Symposia

**SUNDAY, SEPTEMBER 25, 2016 | Symposium 2 | 8:00 – 9:30 am**

## **BALLROOM SALON D**

### *A 360° View of Immunogenicity: Qualitative & Quantitative Assessments to Understand Its Implications on Pharmacokinetics, Safety & Efficacy*

#### **DISCOVERY TRACK**

##### **CO-CHAIRS:**

**Chaitali Passey, PhD**, Senior Research Investigator, Bristol-Myers Squibb Co

**Sumit Rawal, PhD**, Scientist, Regeneron Pharmaceuticals Inc

##### **TARGET AUDIENCE:**

The target audience includes clinical pharmacologists, clinicians, pharmacometricians, regulators and bioanalytical scientists.

##### **GOALS AND OBJECTIVES:**

Following completion of this activity, the learner will be able to:

1. Understand the best practices for reporting and visualization of immunogenicity data;
2. Demonstrate and compare quantitative approaches to assess the impact of immunogenicity on pharmacokinetics;
3. Understand the implications of immunogenicity on safety and efficacy of therapeutic protein products in a clinical setting.

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8:00 – 8:05 am

#### **Session Overview**

*Chaitali Passey, PhD, Senior Research Investigator, Bristol-Myers Squibb Co*

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8:05 – 8:30 am

#### **A Qualitative Look at Immunogenicity Data During Drug Development**

*George R. Gunn III, PhD, Associate Scientific Director, Janssen Research & Development LLC*

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8:30 – 8:55 am

#### **Quantitative Approaches to Assess Immunogenicity During Drug Development of Biologics**

*Leonid Gibiansky, PhD, President, QuantPharm LLC*

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8:55 – 9:20 am

#### **Risk Assessment & Mitigation Strategies for Immunogenicity of Therapeutic Proteins**

*Amy Rosenberg, MD, Supervisory Medical Officer, Div of Therapeutic Proteins, US Food & Drug Administration*

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9:20 – 9:30 am

#### **Q&A**



# ACCP

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## Symposia

SUNDAY, SEPTEMBER 25, 2016 | Symposium 3 | 10:00 am – 12:00 pm

### BALLROOM SALON D

## Helping Advance the Immuno-Oncology Revolution: Trends in Translational Immuno-Oncology

### DISCOVERY & APPLICATION TRACKS

#### CO-CHAIRS:

**Sree Kasichayanula, PhD**, Principal Scientist, Amgen Inc  
**Yu-Nien (Tom) Sun, PhD**, Senior Director, Janssen Research & Development LLC

#### TARGET AUDIENCE:

The target audience includes physicians, pharmacists, researchers and regulators who are seeking to understand translational research in oncology immunotherapy, along with pharmacokinetics/pharmacodynamics (PK/PD) and systems modeling and the future of drug development to treat patients with cancer. The Symposium attendees will be able to learn and appreciate the utility of novel technologies, such as imaging, and their roles, along with PK/PD, in targeted therapy in oncology. Recent translational advances that helped accelerate combination immunotherapy development, along with future outlook in this disease area, will also be covered.

#### GOALS AND OBJECTIVES:

Following completion of this activity, the learner will be able to:

1. Understand recent advances in translational drug development in cancer immunotherapy;
2. Appreciate the utility of imaging in oncology translational models;
3. Conceptualize the differences in combination immunotherapy and the utility of translational models in acceleration of combination oncology development;
4. Compare the role of traditional PK/PD models and recent advances in systems modeling.

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10:00 – 10:10 am

#### Introduction

*Yu-Nien (Tom) Sun, PhD, Senior Director, Janssen Research & Development LLC*

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10:10 – 10:30 am

#### Bench to Bedside: Recent Examples in Translating Medicine in Cancer Drug Development

*Jonathan Benjamin, MD, Medical Director, Amgen Inc*

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10:30 – 10:50 am

#### Understanding the Utility of Imaging in Targeted Drug Delivery: Opportunities & Challenges in Immuno-Oncology

*Bart Hendriks, PhD, Senior Director of Nanoimaging, Merrimack Pharmaceuticals Inc*

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10:50 – 11:10 am

#### Preclinical Models for Defining Efficacy of Immunotherapy Combinations: Mapping the Road for Acceleration to Clinic

*Christina L. Mayer, PharmD, Senior Scientist, Biologics Clinical Pharmacology, Janssen Research & Development LLC*

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11:10 – 11:30 am

#### Advances & the Future Role of Immuno-Oncology Systems Models

*Donald E. Mager, PharmD, PhD, Associate Professor, Univ at Buffalo, State Univ of New York*

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11:30 am – 12:00 pm

#### Panel Discussion



# Symposia

**SUNDAY, SEPTEMBER 25, 2016 | Symposium 4 | 1:30 – 5:30 pm**

## BALLROOM SALON A–C

### *Clinical Development of Biologics: Current Strategy, Challenges & Future Considerations*

#### DISCOVERY TRACK

##### CO-CHAIRS:

**Gaurav Bajaj, PhD**, Senior Research Investigator, Bristol-Myers Squibb Co  
**Ping Zhao, PhD**, Lead, PBPK Program, Div of Pharmacometrics, US Food & Drug Administration

##### TARGET AUDIENCE:

The session will cover the challenges in clinical pharmacology associated with development of early clinical candidates during Phase 1/2 stages. The target audience includes clinical pharmacologists and pharmacometricians from the pharmaceutical and biotech industries and academia, clinicians and regulatory scientists, scientists working on early drug development and graduate students/trainees in pharmaceutical sciences and clinical pharmacology.

##### GOALS AND OBJECTIVES:

Following completion of this activity, the learner will be able to:

1. Understand challenges in clinical development of monoclonal antibodies (mAbs);
2. Discuss dose-optimization strategies of mAbs for pivotal trials and the possible strategies for mAbs that are approved and are being used in combination with another biologic;
3. Discuss challenges related to characterization of non-linear pharmacokinetics of mAbs and the implication on clinical development;
4. Analyze and predict immunogenicity of mAbs and the impact on clinical efficacy and safety;
5. Understand the current status, limitation, challenges and future directions of using physiologically-based pharmacokinetic and pharmacodynamic (PBPK/PD) models in drug development for biologics;
6. Apply PBPK models to predict drug interaction potential for antibody-drug conjugates.

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1:30 – 1:40 pm

#### Introduction

*Gaurav Bajaj, PhD, Senior Research Investigator, Bristol-Myers Squibb Co*

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1:40 – 2:10 pm

#### Clinical Pharmacology Considerations for Biologics: Important Concepts

*Diane R. Mould, PhD, President, Projections Research Inc*

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2:10 – 2:35 pm

#### Communicating Concepts Correctly

*John D. Davis, BPharm, PhD, Senior Director, Regeneron Pharmaceuticals Inc*

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2:35 – 3:00 pm

#### How the Development of Combination Therapy in Biologics Can Be Different Than Monotherapy

*Manish Gupta, PhD, Director, Clinical Pharmacology & Pharmacometrics, Bristol-Myers Squibb Co*

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3:00 – 3:30 pm / Break

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3:30 – 4:00 pm

#### Application of Physiologically-based Pharmacokinetics in Biologics in Drug Development With a Case Example to Predict Disease-mediated Therapeutic Protein Interaction

*Xiling Jiang, PhD, Senior Scientist, Janssen Research & Development LLC*

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4:00 – 4:30 pm

#### Application of Physiologically-based Pharmacokinetic Models to Predict Drug Interactions for Antibody-Drug Conjugates

*Chunze Li, PhD, Senior Scientist, Genentech Inc*

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4:30 – 5:00 pm

#### Regulatory Considerations in Biologics Development

*Hong Zhao, PhD, Master Reviewer of Clinical Pharmacology/ Team Leader, US Food & Drug Administration*

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5:00 – 5:30 pm

#### Panel Discussion



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## Symposia

**SUNDAY, SEPTEMBER 25, 2016 | Symposium 5 | 1:30 – 3:00 pm**

### **BALLROOM SALON D**

## *Addressing Opioid Dependence: Now is the Time*

### **APPLICATION TRACK**

#### **CO-CHAIRS:**

**Lorraine M. Rusch, PhD**, Vice President, Commercial Development, Celerion Inc

**Michael J. Fossler, Jr, PharmD, PhD**, Vice President, Quantitative Sciences, Trevena Inc

#### **TARGET AUDIENCE:**

The target audience includes clinical pharmacologists involved in basic and applied clinical research focused on analgesia management, pharmacists involved in filling and reporting opioid prescriptions, medical directors, chief medical officers of organizations developing new chemical entities for pain management, physicians (both those practicing in the pain management area and those not as familiar) and health economics professionals.

#### **GOALS AND OBJECTIVES:**

Following completion of this activity, the learner will be able to:

1. Understand and delineate common challenges that persons suffering with substance use disorder face while attempting to secure treatment options such as suboxone and/or methadone and counseling programs;
2. Demonstrate a basic understanding of the principles of the neurobiology involved in addictions, treatment options available and basic medical practice towards the management of addiction;
3. Consider the alternative of decriminalizing those who voluntarily commit to substance treatment through novel community policing programs that secure immediate aid for addicts in need;
4. Understand and utilize new programs (training, grants, increased buprenorphine prescribing) proposed by the US Health & Human Services (HHS) and budgeted for 2016 (\$133M in new funding).

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**1:30 – 1:35 pm**

#### **Introduction**

*Michael J. Fossler, Jr, PharmD, PhD, Vice President, Quantitative Sciences, Trevena Inc*

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**1:35 – 1:55 pm**

#### **Addiction: An Imprecise Problem in a World of Precision Medicine**

*Edward M. Sellers, MD, PhD, Professor Emeritus, Pharmaceuticals, Medicine & Psychiatry, Univ of Toronto*

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**1:55 – 2:20 pm**

#### **HHS: Policies to Address Opioid-drug Related Overdose, Death & Dependence**

*Jinhee Lee, PharmD, Senior Pharmacy Advisor, Substance Abuse & Mental Health Svcs Administration*

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**2:20 – 2:40 pm**

#### **Gloucester Police Department Angel Initiative & the Police Assisted Addiction Recovery Initiative (PAARI)**

*Leonard Campanello, MS, Chief of Police, City of Gloucester, MA Police Dept MAFE*

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**2:40 – 3:00 pm**

#### **Panel Discussion**



# Symposia

**SUNDAY, SEPTEMBER 25, 2016 | Symposium 6 | 3:30 – 5:30 pm**

## **BALLROOM SALON D**

### *Treatment of Hepatitis C with Direct-acting Antiviral Drugs: Opportunities & Challenges*

#### **APPLICATION TRACK**

*Offers both CME and CPE Credit*

**UAN #0238-0000-16-005-L01-P**

**ACPE – 2 CONTACT HOURS/APPLICATION-BASED**

*This Symposium is supported in part by an Educational Grant from Merck Sharp & Dohme Corp (A subsidiary of Merck & Co Inc)*

#### **CO-CHAIRS:**

**Vikram Arya, PhD**, Silver Spring, MD

**Shirley Seo, PhD**, Team Leader, Office of Clinical Pharmacology, US Food & Drug Administration

#### **TARGET AUDIENCE:**

The target audience includes physicians, clinical pharmacologists, pharmacists and academic research scientists.

#### **GOALS AND OBJECTIVES:**

The goal of this course is to provide participants with an insight into the various strategies for treatment of Hepatitis C and to discuss the various challenges of treating HCV-infected patients in the era of all oral direct-acting antiviral (DAA) therapies.

Following completion of this activity, the learner will be able to:

1. Understand recent advances in the treatment of Hepatitis C with DAA drugs and identify the various knowledge gaps;
2. Demonstrate knowledge of the role of clinical pharmacology in optimizing the dose and treatment duration of DAAs for various genotypes;
3. Understand the various dosing recommendations of DAAs in some specific populations (for example HIV/HCV co-infected and transplant patients).

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**3:30 – 3:35 pm**

#### **Introduction**

*Vikram Arya, PhD, Silver Spring, MD*

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**3:35 – 4:00 pm**

#### **Challenges Associated with the Use of Direct-acting Antiviral Drugs in Specific Populations**

*Mark Sulkowski, MD, Professor of Medicine, Johns Hopkins Univ School of Medicine*

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**4:00 – 4:25 pm**

#### **Ultra-short Duration Therapy: Reality or Myth?**

*Shyam Kottilli, MD, PhD, Professor of Medicine, Inst of Human Virology, Univ of Maryland*

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**4:25 – 4:50 pm**

#### **Knowledge Gaps in the Development of Direct-acting Antiviral Drugs: How Clinical Pharmacology Has Contributed to Closing Them**

*Shirley Seo, PhD, Team Leader, Office of Clinical Pharmacology, US Food & Drug Administration*

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**4:50 – 5:30 pm**

#### **Panel Discussion**



# ACCP

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## Symposia

**MONDAY, SEPTEMBER 26, 2016 | Symposium 7 | 8:00 am – 12:00 pm**

### **BALLROOM SALON A-C**

## *Establishing Biosimilarity: The European Perception, Experience & Future Trends*

### **DISCOVERY & APPLICATION TRACKS**

*Offers both CME and CPE Credit*

**UAN #0238-9999-16-006-L01-P**

**ACPE – 3.5 CONTACT HOURS/APPLICATION-BASED**

This activity has been planned and implemented in accordance with the accreditation requirements and policies of the Accreditation Council for Continuing Medical Education (ACCME) through the joint providership of the American College of Clinical Pharmacology and the European Federation for Exploratory Medicines Development. The American College of Clinical Pharmacology is accredited by the ACCME to provide continuing medical education for physicians.

### **CO-CHAIRS:**

**Hildegard Sourgens, MD, PhD**, President Elect, European Federation for Exploratory Medicines Development

**Hartmut Derendorf, PhD**, Distinguished Professor & Chair, V. Ravi Chandran Professor in Pharmaceutical Sciences, Univ of Florida

### **TARGET AUDIENCE:**

The target audience includes healthcare professionals who are involved in the research and development of biopharmaceuticals/biosimilars, regulatory affairs (competent authorities; pharmaceutical industry), pharmacovigilance and/or biotech start-ups.

### **GOALS AND OBJECTIVES:**

The goal is for participants to learn from the European Medicines Agency's (EMA) 15-year experience in biosimilars and assess if similar concepts can be adopted in the US.

Following completion of this activity, the learner will be able to:

1. Demonstrate the primary contribution of analytical comparability and its meaning for clinical development;
2. Analyze the European experience with respect to clinical and nonclinical development programs, approval success, post-marketing performance and the failures of European biosimilar programs (as far as these can be made public);
3. Develop the impact of pharmacokinetics/pharmacodynamics to detect differences between a reference medicinal product and a biosimilar;
4. Demonstrate the safety of biologicals and biosimilars based on the European experience;
5. Demonstrate the discriminative power of analytics and pharmacokinetic and/or pharmacodynamic profiles vs Phase 3 trials.

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8:00 – 8:20 am

### **Introduction/EMA & FDA Guidance Review**

*Hildegard Sourgens, MD, PhD, President Elect, European Federation for Exploratory Medicines Development*

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8:20 – 9:00 am

### **How Similar is Similar? The European Biosimilar Quality Experience**

*Paul Declerck, PhD, Professor, Dean of the Faculty of Pharmaceutical Sciences, Univ of Leuven*

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9:00 – 9:40 am

### **Clinical Strategies for Biosimilar Development: A European Perspective**

*Gabriele Dallmann, PhD, Co-founder, Biopharma Excellence GbR*

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9:40 – 10:10 am / **Break**

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10:10 – 10:50 am

### **The European Experience With Safety Testing of Biologicals and Biosimilars**

*Huib Schellekens, MD, PhD, Chair, Professor in Pharmaceutical Biotechnology, Utrecht Univ*

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10:50 – 11:30 am

### **Current Status & Future Trends in Biologics & Biosimilar Development & Approval in the US**

*Hae-Young Ahn, PhD, RAC, Deputy Director, Div of Clinical Pharmacology III, US Food & Drug Administration*

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11:30 am – 12:00 pm

### **Panel Discussion**



# Symposia

**MONDAY, SEPTEMBER 26, 2016 | Symposium 8 | 8:00 – 9:30 am**

## **BALLROOM SALON D**

### *Informing Pediatric Development Programs: Leveraging Big Data*

#### **DISCOVERY TRACK**

##### **CO-CHAIRS:**

**Jeffrey Barrett, PhD**, Vice President, Translational Informatics, Sanofi  
**Lily (Yeruk) Mulugeta, PharmD**, Scientific Lead for Pediatrics, Div of Pharmacometrics, US Food & Drug Administration

##### **TARGET AUDIENCE:**

The target audience includes drug development scientists in both academia and industry, regulators, clinicians, clinical pharmacologists and statisticians.

##### **GOALS AND OBJECTIVES:**

One of the more compelling challenges in pediatric drug development, as well as the consideration on expanded indications in children for existing approved agents, is understanding the pediatric disease progression. Data sources that include large and unstructured formats, ie, Big Data, are available, but their role in pediatric drug development is only at the genesis stage. Sources of Big Data include the electronic medical record (EMR) and large multi-institution administrative databases which consist of information on demographics, laboratory findings, microbiology data, medical order, procedures, surgery and clinical outcomes. The session will explore potential frameworks for how existing data can be used in pediatric drug development to optimize protocol design and enhance patient recruitment. The session will highlight case studies and discuss unique data sources that can be leveraged.

Following completion of this activity, the learner will be able to:

1. Review varying types of data that can be leveraged to support pediatric trial design;
2. Present examples on how efficiency of pediatric trials can be improved using existing data;
3. Discuss the limitations and generalizability of data from EMR as it applies to pediatric drug development.

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**8:00 – 8:15 am**

#### **Introduction to Big Data & Its Relevance for Pediatric Drug Development**

*Jeffrey Barrett, PhD, Vice President, Translational Informatics, Sanofi*

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**8:15 – 8:30 am**

#### **The Value of Historical Electronic Health Records Data to Guide Relevant Clinical Questions Around Pediatric Standard of Care: A Perspective from Cerner**

*Brian Jacobs, MD, Vice President, Chief Medical Information Officer & Chief Information Officer, Children's National Health System*

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**8:30 – 8:45 am**

#### **Combining Bedside & Clinical Research Data to Inform Disease Progression and Outcomes/ Biomarker Selection**

*Diane R. Mould, PhD, President, Projections Research Inc*

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**8:45 – 9:00 am**

#### **Deriving Insight & Value from Electronic Health Records: Opportunities and Challenges of Neonatal Clinical Research in the Big Data Era**

*P. Brian Smith, MD, MHS, MPH, Professor of Pediatrics, Duke Univ Medical Ctr*

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**9:00 – 9:15 am**

#### **Using Existing Data Sources for Advancing Clinical Trials: A Regulatory Perspective**

*Jeffrey Florian, PhD, Team Leader, Div of Pharmacometrics, US Food & Drug Administration*

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**9:15 – 9:30 am**

#### **Panel Discussion**

*(including Brian Jacobs, MD [Children's National Health System], P. Brian Smith, MD, MHS, MPH [Duke Univ Medical Ctr], Anne Zajicek, MD, PharmD [National Inst of Health], Lynne Yao, MD [US Food & Drug Administration], Vikram Sinha, PhD [Merck Research Laboratories] and Diane R. Mould, PhD [Projections Research Inc])*



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## Symposia

MONDAY, SEPTEMBER 26, 2016 | Symposium 9 | 10:00 am – 12:00 pm

### BALLROOM SALON D

## Little Children, Big Challenges: The Problems for Neonatal Drug Trials & the Way Forward

### APPLICATION TRACK

*Offers both CME and CPE Credit*

UAN #0238-0000-16-007-L01-P

ACPE – 2 CONTACT HOURS/APPLICATION-BASED

### CO-CHAIRS:

**Jian Wang, PhD**, Senior Clinical Pharmacologist, US Food & Drug Administration

**John van den Anker, MD, PhD**, Chief, Div of Clinical Pharmacology, Children's National Health System

### TARGET AUDIENCE:

The target audience includes clinical pharmacologists from both pharmaceutical & biotechnology companies and regulatory agencies, pharmacometricians, clinical researchers and drug development scientists who have an interest in applying and/or currently apply principles of pediatric clinical pharmacology to innovate and accelerate drug development for neonatal patients.

### GOALS AND OBJECTIVES:

Following completion of this activity, the learner will be able to:

1. Review the challenges and opportunities in neonatal drug development from a preclinical and clinical pharmacology perspective;
2. Demonstrate how understanding ontogeny and clinical data together inform neonatal dose selection;
3. Discuss approaches that can be used to improve efficiency and feasibility of neonatal trials;
4. Evaluate the use of optimal clinical trial design in neonatal patients;
5. Discuss safety evaluation in neonates;
6. Update participants about the FDA pediatric guidances and the international neonatal consortium.

10:00 – 10:05 am

### Overview: Challenges & Opportunities

*John van den Anker, MD, PhD, Chief, Div of Clinical Pharmacology, Children's National Health System*

10:05 – 10:20 am

### Ontogeny of Drug-metabolizing Enzymes & Drug Transporters: What is Known & Unknown

*J. Steven Leeder, PharmD, PhD, Director, Div of Clinical Pharmacology, Toxicology & Therapeutic Innovation, Children's Mercy Hosp*

10:20 – 10:35 am

### Considerations on Clinical Outcome Measures & Biomarkers: Pediatric Trials Network Experience

*P. Brian Smith, MD, MHS, MPH, Professor of Pediatrics, Duke Univ Medical Ctr*

10:35 – 10:50 am

### Innovative Trial Designs for Neonatal Studies

*Lynne Yao, MD, Director, Div of Pediatric & Maternal Health, US Food & Drug Administration*

10:50 – 11:05 am

### Extrapolation in Neonates: What is the Role of Clinical Pharmacology?

*Ronald J. Portman, MD, Executive Director, Pediatric Therapeutic Area, Novartis Pharmaceuticals Corp*

11:05 – 11:15 am

### Neonatal Safety Studies

*Gilbert J. Burckart, PharmD, Associate Director for Pediatrics, US Food & Drug Administration*

11:15 – 11:30 am

### International Neonatal Consortium Update

*Susan McCune, MD, Deputy Director, Office of Translational Sciences, US Food & Drug Administration*

11:30 am – 12:00 pm

### Panel Discussion

*(including Gerri Baer, MD, Medical Officer/Neonatologist, US Food & Drug Administration)*



# Symposia

MONDAY, SEPTEMBER 26, 2016 | Symposium 10 | 1:30 – 5:30 pm

## BALLROOM SALON A–C

### *Streamlining Clinical Pharmacology Strategies During Early Development: Assessment of Drug-Drug Interactions, Food Effect & QTc*

#### DISCOVERY TRACK

##### CO-CHAIRS:

**Suraj G. Bhansali, MS, PhD**, Manager, Oncology Clinical Pharmacology, Novartis Pharmaceuticals Corp

**Xiao Hu, PhD**, Senior Pharmacometrician, Biogen Inc

##### TARGET AUDIENCE:

The target audience includes clinical pharmacologists, pharmacokineticists and clinicians. The international scientific community in academia, the pharmaceutical & biotechnology industries or regulatory authorities associated with clinical drug development will also be particularly interested in this topic based on the common challenges faced during development of oncology compounds.

##### GOALS AND OBJECTIVES:

Following completion of this activity, the learner will be able to:

1. Understand challenges specific to oncology clinical drug development;
2. Evaluate the timing of clinical pharmacology studies for oncology compounds in reference to the normal clinical development plan and discuss benefits of doing studies at an early stage;
3. Discuss strategies for early assessment of food effect, QTc and drug-drug interactions (DDI);
4. Discuss considerations to streamline proarrhythmic risk assessment during early clinical development.

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1:30 – 1:40 pm

#### Introduction

*Suraj G. Bhansali, MS, PhD, Manager, Oncology Clinical Pharmacology, Novartis Pharmaceuticals Corp and Xiao Hu, PhD, Senior Pharmacometrician, Biogen Inc*

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1:40 – 2:10 pm

#### **An Overview of Early Clinical Pharmacology Studies: Assessment of Drug-Drug Interactions, Food Effect & QTc in an Oncology Setting**

*Suraj G. Bhansali, MS, PhD, Manager, Oncology Clinical Pharmacology, Novartis Pharmaceuticals Corp*

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2:10 – 2:35 pm

#### **Early Assessment of Drug-Drug Interaction Potential in Combination Clinical Trials**

*Konstantine W. Skordos, PhD, Director, Clinical Pharmacology, Translational Clinical Oncology, Novartis Pharmaceuticals Corp*

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2:35 – 3:00 pm

#### **Food Effects in Early Cancer Drug Development: More Than Meets the Eye**

*Lawrence J. Lesko, PhD, Clinical Professor & Director, Ctr for Pharmacometrics & Systems Pharmacology, Univ of Florida*

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3:00 – 3:30 pm / Break

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3:30 – 4:00 pm

#### **It's Time to Revise ICH E14 Guidance: The History, Opportunities, Challenges & Directions of QTc Analysis**

*Christine Garnett, PharmD, Clinical Analyst, US Food & Drug Administration*

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4:00 – 4:30 pm

#### **Key Considerations in Study Design & Analysis Methods of New Drugs' QT Assessment Using a Phase 1 Study**

*Jiang Liu, PhD, Scientific Lead for QT, US Food & Drug Administration*

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4:30 – 5:00 pm

#### **Using Concentration-QTc Analysis to Obtain a Waiver for TQT Study**

*Cara Nelson, PhD, Clinical Pharmacologist II, Gilead Sciences Inc*

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5:00 – 5:30 pm

#### **Panel Discussion**



# ACCP

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## Symposia

MONDAY, SEPTEMBER 26, 2016 | Symposium 11 | 1:30 – 3:00 pm

### BALLROOM SALON D

## Cutting-edge Abstract Presentations

#### DISCOVERY & APPLICATION TRACKS

*Offers both CME and CPE Credit*

UAN #0238-0000-16-008-L01-P

ACPE – 1.5 CONTACT HOURS/APPLICATION-BASED

#### CO-CHAIRS:

**Lawrence J. Cohen, PharmD**, Professor & Coordinator of Interprofessional Education, Univ of North Texas System Coll of Pharmacy

**Walter K. Kraft, MD**, Professor, Thomas Jefferson Univ

**Amalia M. Issa, PhD**, Professor & Chair, Personalized Medicine & Targeted Therapeutics, Univ of the Sciences

#### TARGET AUDIENCE:

The target audience includes physicians, pharmacists and clinical pharmacologists involved in basic and applied clinical research who are interested in state-of-the-art investigations.

#### GOALS AND OBJECTIVES:

Following completion of this activity, the learner will be able to:

1. Describe at least two of the abstracts from a curated list of the top abstracts submitted for the 2016 ACCP Annual Meeting;
2. Identify a novel area or focus of clinical pharmacology research;
3. Interact with the authors of multiple cutting-edge abstracts from a variety of disciplines.

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1:30 – 1:40 pm

#### Introduction & Abstract Award Winner Announcement

*Lawrence J. Cohen, PharmD, Professor & Coordinator of Interprofessional Education, Univ of North Texas System Coll of Pharmacy, Walter K. Kraft, MD, Professor, Thomas Jefferson Univ and Amalia M. Issa, PhD, Professor & Chair, Personalized Medicine & Targeted Therapeutics, Univ of the Sciences*

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1:40 – 1:49 pm

#### Abstract #1

*Wayne A. Colburn Memorial Award (5 minute presentation; 3 minutes of questions)*

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1:50 – 1:59 pm

#### Abstract #2

*New Member Abstract Award (5 minute presentation; 3 minutes of questions)*

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2:00 – 2:09 pm

#### Abstract #3

*(5 minute presentation; 3 minutes of questions)*

---

2:10 – 2:19 pm

#### Abstract #4

*(5 minute presentation; 3 minutes of questions)*

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2:20 – 2:29 pm

#### Abstract #5

*(5 minute presentation; 3 minutes of questions)*

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2:30 – 2:39 pm

#### Abstract #6

*(5 minute presentation; 3 minutes of questions)*

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2:40 – 2:49 pm

#### Abstract #7

*(5 minute presentation; 3 minutes of questions)*

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2:50 – 2:59 pm

#### Abstract #8

*(5 minute presentation; 3 minutes of questions)*

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3:00 pm

#### Wrap Up



# Symposia

**MONDAY, SEPTEMBER 26, 2016 | Symposium 12 | 3:30 – 5:30 pm**

## **BALLROOM SALON D**

### *Rethinking Clinical Pharmacology-related Labeling for Improved Utility & Comprehension*

#### **DISCOVERY & APPLICATION TRACKS**

*Offers both CME and CPE Credit*

**UAN #0238-0000-16-009-L03-P**

**ACPE – 2 CONTACT HOURS/APPLICATION-BASED**

#### **CO-CHAIRS:**

**Joseph A. Grillo, PharmD**, Associate Director for Labeling & Health Communications, US Food & Drug Administration

**Julie Bullock, PharmD**, Senior Director, d3 Medicine LLC

#### **TARGET AUDIENCE:**

The target audience includes scientists in the pharmaceutical industry, healthcare providers and regulatory professionals.

#### **GOALS AND OBJECTIVES:**

Following completion of this activity, the learner will be able to:

1. Present stakeholder experiences (eg, industry, academia/clinical practice, FDA, cognitive scientist) regarding clinical pharmacology-related information in labeling;
2. Explore different labeling formats (eg, tables, figures, structured text) to further enhance the presentation of clinical pharmacology information in labeling;
3. Provide an overview of key principles included in the FDA Clinical Pharmacology Section Labeling guidance (under revision).

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**3:30 – 3:40 pm**

#### **Introduction**

*Julie Bullock, PharmD, Senior Director, d3 Medicine LLC*

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**3:40 – 4:00 pm**

#### **Developing Clinical Pharmacology Labeling for Improved Utility & Comprehension: Industry Perspective**

*Dora W. Cohen, BA, MA, Executive Director, Global Labeling, Amgen Inc*

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**4:00 – 4:20 pm**

#### **Strategies for Enhancing Quality, Utility & Clarity in Clinical Pharmacology Labeling: A Regulatory Perspective**

*Joseph A. Grillo, PharmD, Associate Director for Labeling & Health Communications, US Food & Drug Administration*

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**4:20 – 4:40 pm**

#### **Utility & Comprehension of Clinical Pharmacology Labeling: A Healthcare Provider Perspective**

*Patricia W. Slattum, PharmD, PhD, Professor of Pharmacotherapy & Outcomes Science, Virginia Commonwealth Univ*

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**4:40 – 5:00 pm**

#### **Enhanced Displays of Clinical Pharmacology Information: Effects on Attention, Comprehension & Memory**

*Ruth S. Day, PhD, Director, Medical Cognition Laboratory, Psychology & Neuroscience, Duke Univ*

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**5:00 – 5:30 pm**

#### **Panel Discussion**

*(including Eric Brodsky, MD [US Food & Drug Administration])*



# ACCP

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## Symposia

**TUESDAY, SEPTEMBER 27, 2016 | Symposium 13 | 8:00 am – 12:00 pm**

### BALLROOM SALON A-C

## Orphan Drug Development in Adults & Pediatrics: Industry, Academia & Regulatory Perspectives

#### DISCOVERY TRACK

*Offers both CME and CPE Credit*

UAN #0238-0000-16-010-L01-P

ACPE – 3.5 CONTACT HOURS/APPLICATION-BASED

*This Symposium is supported in part by an Educational Grant from Amicus Therapeutics*

#### CO-CHAIRS:

**Vijay Ivaturi, PhD**, Assistant Professor, Univ of Maryland Baltimore

**Venkatesh Atul Bhattaram, PhD**, Senior Staff Fellow, Office of Clinical Pharmacology, US Food & Drug Administration

#### TARGET AUDIENCE:

The target audience includes researchers, clinicians, entrepreneurs and academic technology transfer staff interested in orphan drug development.

#### GOALS AND OBJECTIVES:

Following completion of this activity, the learner will be able to:

1. Describe state-of-the-art research on orphan drug development;
2. Understand the regulatory pathways available for orphan drug development;
3. Facilitate greater research collaboration and creation of learning communities across disciplines, sectors and initiatives;
4. Discuss entrepreneurial opportunities and mechanisms to innovate and excel in orphan drug development.

8:00 – 8:15 am

#### Introduction

*Vijay Ivaturi, PhD, Assistant Professor, Univ of Maryland Baltimore and Venkatesh Atul Bhattaram, PhD, Senior Staff Fellow, Office of Clinical Pharmacology, US Food & Drug Administration*

8:15 – 8:40 am

#### FDA Flexibility in Facilitating Drug Development in Rare Diseases

*Devanand Jillapalli, MD, Medical Officer, Office of Orphan Drug Products Development, US Food & Drug Administration*

8:40 – 9:10 am

#### Can Universities Make a Difference in the Development of Orphan Drugs?

*James Cloyd III, PharmD, Professor, Neurology & Experimental & Clinical Pharmacology, Univ of Minnesota, Coll of Pharmacy*

9:10 – 9:35 am

#### Drug Development in Rare Neurological Diseases: Clinical Pharmacology Perspective

*Bilal S. AbuAsal, PhD, Clinical Pharmacologist, US Food & Drug Administration*

9:35 – 10:00 am / Break

10:00 – 10:30 am

#### Bootstrapping Orphan Drug Development

*Zachary Rome, BS, MST, Co-founder & Executive Vice President, Patagonia Pharmaceuticals LLC*

10:30 – 11:00 am

#### Statistical Issues In Rare Disease Clinical Trial Design

*Anindya Roy, PhD, Professor, Univ of Maryland Baltimore County*

11:00 am – 12:00 pm

#### Panel Discussion



# Symposia

**TUESDAY, SEPTEMBER 27, 2016 | Symposium 14 | 8:00 – 9:30 am**

## **BALLROOM SALON D**

### *Clinical Applications of Physiologically-based Pharmacokinetics/ Pharmacodynamics for Pediatrics: Academic, Industry & Regulatory Perspectives*

#### **DISCOVERY TRACK**

##### **CO-CHAIRS:**

**Jennifer Sheng, PhD, PharmD**, Associate Director, Clinical Pharmacology & Pharmacometrics, Bristol-Myers Squibb Co

**Diansong Zhou, PhD**, Director, Quantitative Clinical Pharmacology, AstraZeneca plc

##### **TARGET AUDIENCE:**

The target audience includes clinical pharmacologists in the pharmaceutical industry and reviewers at the US Food & Drug Administration.

##### **GOALS AND OBJECTIVES:**

Following completion of this activity, the learner will be able to:

1. Describe the opportunities and challenges in pediatric physiologically-based pharmacokinetic and pharmacodynamic (PBPK/PD) modeling;
2. Illustrate how pediatric PBPK modeling can be used to answer clinical development questions with case examples.

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8:00 – 8:10 am

##### **Introduction**

*Jennifer Sheng, PhD, PharmD, Associate Director, Clinical Pharmacology & Pharmacometrics, Bristol-Myers Squibb Co*

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8:10 – 8:30 am

##### **Overview of Development of Integrated Pediatric Physiologically-based Pharmacokinetic/ Pharmacodynamic Models**

*Trevor N. Johnson, PhD, Principal Scientist, Simcyp Ltd*

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8:30 – 8:50 am

##### **Pediatric PBPK Strategies & Tools During Drug Development**

*Tycho Heimbach, PhD, Director, Drug Metabolism & Pharmacokinetics, Novartis Pharmaceuticals Corp*

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8:50 – 9:10 am

##### **Pediatric PBPK Modeling: Regulatory Experience & Perspective**

*Ping Zhao, PhD, Lead, PBPK Program, Div of Pharmacometrics, US Food & Drug Administration*

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9:10 – 9:30 am

##### **Panel Discussion**



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## Symposia

**TUESDAY, SEPTEMBER 27, 2016 | Symposium 15 | 10:00 – 11:45 am**

### BALLROOM SALON D

## Combination Therapy in Oncology: Challenges & Strategies in Clinical Pharmacology

### APPLICATION TRACK

#### CO-CHAIRS:

**Yilong Zhang, PhD**, Principal Scientist, Amgen Inc

**Satyendra Suryawanshi, PhD**, Associate Director, Bristol-Myers Squibb Co

#### TARGET AUDIENCE:

The target audience includes clinical pharmacologists, physicians and healthcare professionals, oncology researchers in both industry and academics and graduate students in pharmaceutical sciences and clinical pharmacology.

#### GOALS AND OBJECTIVES:

Following completion of this activity, the learner will be able to:

1. Understand the combination requirements and challenges in small- and large-molecule oncology drug development;
2. Utilize clinical pharmacology strategies to optimize dose selection in oncology combination trials;
3. Apply mechanistic models including physiologically-based pharmacokinetics (PBPK) to address complex clinical pharmacology issues;
4. Gain regulatory insight on current status of supporting regulatory decisions using PBPK results, including the use of PBPK data in product labels.

10:00 – 10:10 am

#### Introduction

*Yilong Zhang, PhD, Principal Scientist, Amgen Inc*

10:10 – 10:30 am

#### Clinical Perspectives in Combination Requirements & Challenges in Small- & Large- molecule Oncology Drug Development

*R. Donald Harvey, PharmD, Associate Professor & Director,  
Phase I Clinical Trials Program, Winship Cancer Inst, Emory Univ*

10:30 – 10:50 am

#### Application of Physiologically-based Pharmacokinetic Modeling to Facilitate Dose Optimization in Combination Therapy

*Karen Rowland Yeo, PhD, Senior Scientific Advisor, Simcyp Ltd*

10:50 – 11:10 am

#### Clinical Pharmacology Considerations in Oncology Combination Studies

*Sree Kasichayanula, PhD, Principal Scientist, Amgen Inc*

11:10 – 11:30 am

#### Utility of PBPK in the Oncology Drug Development Experience of Using Quantitative Data Generated from Clinical Pharmacology Programs in Regulatory Decision Making & Product Labels

*Ping Zhao, PhD, Lead, PBPK Program, Div of Pharmacometrics,  
US Food & Drug Administration*

11:30 – 11:45 am

#### Panel Discussion



# Symposia

**TUESDAY, SEPTEMBER 27, 2016 | Symposium 16 | 1:30 – 5:30 pm**

## **BALLROOM SALON A–C**

### *Clinical Pharmacology Strategies in Precision Medicine-based Drug Development & Preventive Medicine*

#### **DISCOVERY & APPLICATION TRACKS**

##### **CO-CHAIRS:**

**Priyanka Jadhav, PhD**, Translational Clinical Pharmacologist, CRC Pharma LLC

**Jinshan Shen, PhD**, Senior Director, Drug Metabolism & Pharmacokinetics, Radius Health Inc

**Manoj P. Jadhav, PhD**, Translational Clinical Pharmacologist, CRC Pharma LLC

##### **TARGET AUDIENCE:**

The target audience includes healthcare professionals, including physicians, clinical pharmacologists and basic scientists who are involved in drug development and research in industry and academics. The course is also applicable to students pursuing their MD, PhD or PharmD.

##### **GOALS AND OBJECTIVES:**

Following completion of this activity, the learner will be able to:

1. Understand recent advances in research and development in the area of precision medicine;
2. Implement recent advances related to research and treatment using a personalized medicine approach, as appropriate;
3. Understand and discuss the role of genetics, pharmacogenomics, Big Data and regulations in the implementation of this concept of precision medicine.

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1:30 – 1:40 pm

#### **Introduction**

*Jinshan Shen, PhD, Senior Director, Drug Metabolism & Pharmacokinetics, Radius Health Inc*

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1:40 – 2:05 pm

#### **Why Clinical Pharmacology is Positioned Well to Excel in Precision Medicine**

*Jogarao V. Gobburu, PhD, MBA, Professor, Univ of Maryland*

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2:05 – 2:30 pm

#### **Clinical Pharmacology of Precision Medicine Treating Cancer Patients**

*Qi Liu, PhD, Clinical Pharmacology Team Leader, US Food & Drug Administration*

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2:30 – 3:00 pm

#### **Genetics & Precision Medicine: The Way Forward!**

*Prasun Mishra, MSc, PhD, Scientist/Principal Investigator, Genentech Inc*

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3:00 – 3:30 pm / **Break**

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3:30 – 3:55 pm

#### **Multi-domain Inference in Healthcare**

*Mattia Proserpi, PhD, Associate Professor, Univ of Florida*

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3:55 – 4:20 pm

#### **Clinical Pharmacology in Cardiovascular Precision Medicine**

*Manoj P. Jadhav, PhD, Translational Clinical Pharmacologist, CRC Pharma LLC*

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4:20 – 4:45 pm

#### **Pharmacogenomics in Drug Development of Precision Medicine – An FDA Perspective**

*Michael Pacanowski, PharmD, MPH, Associate Director for Genomics & Targeted Therapy, US Food & Drug Administration*

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4:45 – 5:30 pm

#### **Panel Discussion**



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## Symposia

**TUESDAY, SEPTEMBER 27, 2016 | Symposium 17 | 1:30 – 5:30 pm**

### BALLROOM SALON D

## Reproducible Visualization & Data Analysis With R

### DISCOVERY TRACK

**Offers both CME and CPE Credit**

**UAN #0238-0000-16-018-L-01-P**

**ACPE – 3.5 CONTACT HOURS/APPLICATION-BASED**

#### CHAIR:

**Devin Pastoor, MTOX**, Software Engineer & PhD Candidate, Ctr for Translational Medicine, Schools of Pharmacy & Medicine, Univ of Maryland Baltimore

#### TARGET AUDIENCE:

The target audience includes persons that use R for data management, statistical analysis or visualization. Attendees should have basic R exposure (read in a dataset, basic data management), with varied examples, material and solutions catered to beginner, intermediate and advanced users.

#### GOALS AND OBJECTIVES:

Following completion of this activity, the learner will be able to:

1. Use best practices in quickly managing, analyzing and visualizing data in a reproducible fashion;
2. Use hands-on examples to introduce and explore a data management pipeline that leverages best-in-class R packages for easy-to-use, powerful workflows.

1:30 – 1:45 pm

#### Introduction

*Devin Pastoor, MTOX, Software Engineer & PhD Candidate, Ctr for Translational Medicine, Schools of Pharmacy & Medicine, Univ of Maryland Baltimore*

1:45 – 2:00 pm

#### Introduction to a Reproducible R Workflow with Rstudio, Rmarkdown

*Devin Pastoor, MTOX, Software Engineer & PhD Candidate, Ctr for Translational Medicine, Schools of Pharmacy & Medicine, Univ of Maryland Baltimore*

2:00 – 2:20 pm

#### Introduction to ggplot2 for Data Visualization

*Kaori Ito, PhD, Director, Pfizer Inc*

2:20 – 2:45 pm

#### Hands-on Activity

*Kaori Ito, PhD, Director, Pfizer Inc*

2:45 – 3:00 pm

#### Solutions Demonstration

*Kaori Ito, PhD, Director, Pfizer Inc*

3:00 – 3:30 pm / Break

3:30 – 4:00 pm

#### Introduction to Data Management with dplyr

*Devin Pastoor, MTOX, Software Engineer & PhD Candidate, Ctr for Translational Medicine, Schools of Pharmacy & Medicine, Univ of Maryland Baltimore*

4:00 – 4:15 pm

#### Additional dplyr & Introduction to tidyr

*Vijay Ivaturi, PhD, Assistant Professor, Univ of Maryland Baltimore*

4:15 – 5:00 pm

#### Hands-on Activity Using dplyr, tidyr & ggplot2

*Vijay Ivaturi, PhD, Assistant Professor, Univ of Maryland Baltimore*

5:00 – 5:30 pm

#### Wrap Up, Q&A, Additional Resources

*Devin Pastoor, MTOX, Software Engineer & PhD Candidate, Ctr for Translational Medicine, Schools of Pharmacy & Medicine, Univ of Maryland Baltimore*

# Faculty

Last Name	First Name	Activity	Affiliation
Abernethy	Darrell R.	Pre-meeting Workshop 3	Associate Director for Drug Safety, Office of Clinical Pharmacology, US Food & Drug Administration
AbuAsal	Bilal S.	Symposium 13	Clinical Pharmacologist, US Food & Drug Administration
Ahn	Hae-Young	Symposium 7	Deputy Director, Div of Clinical Pharmacology III, US Food & Drug Administration
Amur	Shashi	Pre-meeting Workshop 2	Scientific Lead, Biomarker Qualification Program, US Food & Drug Administration
Arya	Vikram	Symposium 6	Silver Spring, MD
Baer	Gerri	Symposium 9	Medical Officer/Neonatologist, US Food & Drug Administration
Bajaj	Gaurav	Symposium 4	Senior Research Investigator, Bristol-Myers Squibb Co
Barrett	Jeffrey	Symposium 8	Vice President, Translational Informatics, Sanofi
Benjamin	Jonathan	Symposium 3	Medical Director, Amgen Inc
Bergman	Kimberly L.	Symposium 1	Lead Pharmacologist, US Food & Drug Administration
Bhansali	Suraj G.	Symposium 10	Manager, Oncology Clinical Pharmacology, Novartis Pharmaceuticals Corp
Bhattacharya	Indranil	Pre-meeting Workshop 1	Director, Pfizer Inc
Bhattaram	Venkatesh Atul	Symposium 13	Senior Staff Fellow, Office of Clinical Pharmacology, US Food & Drug Administration
Brar	Satjit	Pre-meeting Workshop 4	Associate Director, Clinical Pharmacology, Pfizer Inc
Brodsky	Eric	Symposium 12	Associate Director, Labeling Development Team, Office of New Drugs, US Food & Drug Administration
Bullock	Julie	Symposium 12	Senior Director, d3 Medicine LLC
Burckart	Gilbert J.	Pre-meeting Workshop 3 & Symposium 9	Associate Director for Pediatrics, US Food & Drug Administration
Campanello	Leonard	Symposium 5	Chief of Police, City of Gloucester, MA Police Dept MAFE
Cherala	Ganesh	Pre-meeting Workshop 2	Research Scientist, Research Technologies, Novo Nordisk Inc
Chow	Andrew T.	Symposium 1	Executive Director, Amgen Inc
Cloyd III	James	Symposium 13	Professor, Neurology & Experimental & Clinical Pharmacology, Univ of Minnesota, Coll of Pharmacy
Cohen	Dora W.	Symposium 12	Executive Director, Global Labeling, Amgen Inc
Cohen	Lawrence J.	Symposium 11	Professor & Coordinator of Interprofessional Education, Univ of North Texas System Coll of Pharmacy
Corey	Alfred	Symposium 1	Consultant, AC Pharmaco LLC
Dallmann	Gabriele	Symposium 7	Co-founder, Biopharma Excellence GbR
Davis	John D.	Symposium 4	Senior Director, Regeneron Pharmaceuticals Inc
Day	Ruth S.	Symposium 12	Director, Medical Cognition Laboratory, Psychology & Neuroscience, Duke Univ



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## Faculty

Last Name	First Name	Activity	Affiliation
Declerck	Paul	Symposium 7	Professor, Dean of the Faculty of Pharmaceutical Sciences, Univ of Leuven
Derendorf	Hartmut	Symposium 7	Distinguished Professor & Chair, V. Ravi Chandran Professor in Pharmaceutical Sciences, Univ of Florida
Doncel	Gustavo F.	Pre-meeting Workshop 2	Scientific Director, CONRAD & Professor of Obstetrics & Gynecology, Eastern Virginia Medical School
Duff	Sir Gordon W.	Pre-meeting Workshop 1	Professor & Chair of the Biotechnology & Biological Sciences Research Council, St Hilda's Coll, Univ of Oxford
Earp	Justin C.	Pre-meeting Workshop 4	Pharmacometrics Reviewer, US Food & Drug Administration
Eissing	Thomas	Pre-meeting Workshop 3	Head of Systems Pharmacology CV, Bayer Technology Svcs GmbH
Faggioni	Raffaella	Pre-meeting Workshop 1	Senior Director, Clinical Pharmacology & DMPK, MedImmune LLC/AstraZeneca plc
Florian	Jeffry	Symposium 8	Team Leader, Div of Pharmacometrics, US Food & Drug Administration
Fossler, Jr	Michael J.	Symposium 5	Vice President, Quantitative Sciences, Trevena Inc
Garnett	Christine	Symposium 10	Clinical Analyst, US Food & Drug Administration
Gibiansky	Leonid	Symposium 2	President, QuantPharm LLC
Gobburu	Jogarao V.	Symposium 16	Professor, Univ of Maryland
Goel	Varun	Pre-meeting Workshop 4	Fellow, Clinical Pharmacology, Novartis Inst for Biomedical Research
Golden	Adam	Pre-meeting Workshop 3	Professor, Internal Medicine, Univ of Central Florida, Coll of Medicine, Associate Chief of Staff, Geriatrics and Extended Care, Orlando Veterans Affairs Medical Ctr
Grillo	Joseph A.	Symposium 12	Associate Director for Labeling & Health Communications, US Food & Drug Administration
Gunn, III	George R.	Symposium 2	Associate Scientific Director, Janssen Research & Development LLC
Gupta	Manish	Symposium 4	Director, Clinical Pharmacology & Pharmacometrics, Bristol-Myers Squibb Co
Harirforoosh	Sam	Pre-meeting Workshop 2	Associate Professor, Dept of Pharmaceutical Sciences, East Tennessee State Univ, Gatton Coll of Pharmacy
Harvey	R. Donald	Symposium 15	Associate Professor & Director, Phase I Clinical Trials Program, Winship Cancer Inst, Emory Univ
Heimbach	Tycho	Symposium 14	Director, Drug Metabolism & Pharmacokinetics, Novartis Pharmaceuticals Corp
Hendriks	Bart	Symposium 3	Senior Director of Nanoimaging, Merrimack Pharmaceuticals Inc
Hendrix	Craig W.	Pre-meeting Workshop 2	Wellcome Professor & Director, Johns Hopkins Univ School of Medicine
Hu	Xiao	Symposium 10	Senior Pharmacometrician, Biogen Inc
Issa	Amalia M.	Symposium 11	Professor & Chair, Personalized Medicine & Targeted Therapeutics, Univ of the Sciences
Ito	Kaori	Symposium 17	Director, Pfizer Inc

# Faculty

Last Name	First Name	Activity	Affiliation
Ivaturi	Vijay	Symposia 13 & 17	Assistant Professor, Univ of Maryland Baltimore
Jacobs	Brian	Symposium 8	Vice President, Chief Medical Information Officer & Chief Information Officer, Children's National Health System
Jadhav	Manoj P.	Symposium 16	Translational Clinical Pharmacologist, CRC Pharma LLC
Jadhav	Priyanka	Symposium 16	Translational Clinical Pharmacologist, CRC Pharma LLC
Jiang	Xiling	Symposium 4	Senior Scientist, Janssen Research & Development LLC
Jillapalli	Devanand	Symposium 13	Medical Officer, Office of Orphan Drug Products Development, US Food & Drug Administration
Johnson	Trevor N.	Symposium 14	Principal Scientist, Simcyp Ltd
Karlsson	Mats O.	Pre-meeting Workshop 4	Professor, Dept of Pharmaceutical Biosciences, Uppsala Univ
Kasichayanula	Sree	Symposia 3 & 15	Principal Scientist, Amgen Inc
Kottlil	Shyam	Symposium 6	Professor of Medicine, Inst of Human Virology, Univ of Maryland
Kraft	Walter K.	Symposium 11	Professor, Thomas Jefferson Univ
Kumar	Parag	Pre-meeting Workshop 2	Director, Clinical Pharmacokinetics Research Lab, National Inst of Health
Lau	S.W. Johnny	Pre-meeting Workshop 3	Senior Clinical Pharmacologist, US Food & Drug Administration
Lee	Jinhee	Symposium 5	Senior Pharmacy Advisor, Substance Abuse & Mental Health Svcs Administration
Leeder	J. Steven	Symposium 9	Director, Div of Clinical Pharmacology, Toxicology & Therapeutic Innovation, Children's Mercy Hosp
Lesko	Lawrence J.	Symposium 10	Clinical Professor & Director, Ctr for Pharmacometrics & Systems Pharmacology, Univ of Florida
Li	Chunze	Pre-meeting Workshop 1 & Symposium 4	Senior Scientist, Genentech Inc
Liu	Jiang	Symposium 10	Scientific Lead for QT, US Food & Drug Administration
Liu	Qi	Symposium 16	Clinical Pharmacology Team Leader, US Food & Drug Administration
Ma	Lian	Symposium 1	Pharmacometrics Reviewer; US Food & Drug Administration
Mager	Donald E.	Symposium 3	Associate Professor, Univ at Buffalo, State Univ of New York
Marathe	Anshu	Pre-meeting Workshop 4	Team Leader, Div of Clinical Pharmacology II, US Food & Drug Administration
Mayer	Christina L.	Symposium 3	Senior Scientist, Biologics Clinical Pharmacology, Janssen Research & Development LLC
McCune	Susan	Symposium 9	Deputy Director, Office of Translational Sciences, US Food & Drug Administration
Mehrotra	Nitin	Symposium 1	Team Leader, Div of Pharmacometrics, US Food & Drug Administration
Meibohm	Bernd	Pre-meeting Workshop 1	Professor & Associate Dean, Univ of Tennessee Health Science Ctr, Coll of Pharmacy



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## Faculty

Last Name	First Name	Activity	Affiliation
Mishra	Prasun	Symposium 16	Scientist/Principal Investigator, Genentech Inc
Moorman	Jonathan P.	Pre-meeting Workshop 2	Professor, East Tennessee State Univ, Quillen Coll of Medicine
Mould	Diane R.	Symposia 4 & 8	President, Projections Research Inc
Mulugeta	Lily (Yeruk)	Symposium 8	Scientific Lead for Pediatrics, Div of Pharmacometrics, US Food & Drug Administration
Nelson	Cara	Symposium 10	Clinical Pharmacologist II, Gilead Sciences Inc
Pacanowski	Michael	Symposium 16	Associate Director for Genomics & Targeted Therapy, US Food & Drug Administration
Passey	Chaitali	Symposium 2	Senior Research Investigator, Bristol-Myers Squibb Co
Pastoor	Devin	Symposium 17	Software Engineer & PhD Candidate, Ctr for Translational Medicine, Schools of Pharmacy & Medicine, Univ of Maryland Baltimore
Portman	Ronald J.	Symposium 9	Executive Director, Pediatric Therapeutic Area, Novartis Pharmaceuticals Corp
Powell	Andrea M.	Symposium 1	Pharmacologist, Counter-terrorism & Emergency Coordination, US Food & Drug Administration
Prosperi	Mattia	Symposium 16	Associate Professor, Univ of Florida
Rahman	Atiqur	Pre-meeting Workshop 4	Director, Div of Clinical Pharmacology V, US Food & Drug Administration
Rawal	Sumit	Symposium 2	Scientist, Regeneron Pharmaceuticals Inc
Rome	Zachary	Symposium 13	Co-founder & Executive Vice President, Patagonia Pharmaceuticals LLC
Rosenberg	Amy	Symposium 2	Supervisory Medical Officer, Div of Therapeutic Proteins, US Food & Drug Administration
Rowland Yeo	Karen	Symposium 15	Senior Scientific Advisor, Simcyp Ltd
Roy	Anindya	Symposium 13	Professor, Univ of Maryland Baltimore County
Rusch	Lorraine M.	Symposium 5	Vice President, Commercial Development, Celerion Inc
Schellekens	Huub	Symposium 7	Chair, Professor in Pharmaceutical Biotechnology, Utrecht Univ
Schlender	Jan-Frederik	Pre-meeting Workshop 3	Pharmacist, Scientist Systems Pharmacology, Bayer Technology Svcs GmbH
Sellers	Edward M.	Symposium 5	Professor Emeritus, Pharmaceuticals, Medicine & Psychiatry, Univ of Toronto
Seo	Shirley	Symposium 6	Team Leader, Office of Clinical Pharmacology, US Food & Drug Administration
Shen	Jinshan	Symposium 16	Senior Director, Drug Metabolism & Pharmacokinetics, Radius Health Inc
Sheng	Jennifer	Symposium 14	Associate Director, Clinical Pharmacology & Pharmacometrics, Bristol-Myers Squibb Co

# Faculty

Last Name	First Name	Activity	Affiliation
Shi	Rong	Pre-meeting Workshop 1	Clinical Pharmacology Lead, Bristol-Myers Squibb Co
Sinha	Vikram	Symposium 8	Associate Vice President, Head Quantitative Pharmacology, Merck Research Laboratories
Skordos	Konstantine W.	Pre-meeting Workshop 4 & Symposium 10	Director, Clinical Pharmacology, Translational Clinical Oncology, Novartis Pharmaceuticals Corp
Slattum	Patricia W.	Pre-meeting Workshop 3 & Symposium 12	Professor of Pharmacotherapy & Outcomes Science, Virginia Commonwealth Univ
Smith	P. Brian	Symposia 8 & 9	Professor of Pediatrics, Duke Univ Medical Ctr
Sourgens	Hildegard	Symposium 7	President Elect, European Federation for Exploratory Medicines Development
Stegemann	Sven	Pre-meeting Workshop 3	Professor, Graz Univ of Technology
Sulkowski	Mark	Symposium 6	Professor of Medicine, Johns Hopkins Univ School of Medicine
Sun	Yu-Nien (Tom)	Symposium 3	Senior Director, Janssen Research & Development LLC
Suryawanshi	Satyendra	Symposium 15	Associate Director, Bristol-Myers Squibb Co
van den Anker	John	Symposium 9	Chief, Div of Clinical Pharmacology, Children's National Health System
Wang	Jian	Symposium 9	Senior Clinical Pharmacologist, US Food & Drug Administration
Wang	Yow-Ming C.	Pre-meeting Workshop 1	Clinical Pharmacology (Biologics) Team Leader, US Food & Drug Administration
Yang	Zheng	Pre-meeting Workshop 1	Director, Bristol-Myers Squibb Co
Yao	Lynne	Symposia 8 & 9	Director, Div of Pediatric & Maternal Health, US Food & Drug Administration
Zajicek	Anne	Symposium 8	Branch Chief, National Inst of Health
Zhang	Yilong	Symposium 15	Principal Scientist, Amgen Inc
Zhao	Hong	Symposium 4	Master Reviewer of Clinical Pharmacology/Team Leader, US Food & Drug Administration
Zhao	Ping	Symposia 4, 14 & 15	Lead, PBPK Program, Div of Pharmacometrics, US Food & Drug Administration
Zhou	Diansong	Symposium 14	Director, Quantitative Clinical Pharmacology, AstraZeneca plc
Zhou	Honghui	Pre-meeting Workshop 1	Senior Director & Janssen Fellow, Janssen Research & Development LLC



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- **Discounted registration for the ACCP Annual Meeting**, your source for current, interprofessional ACCME & ACPE-accredited Continuing Education programs in a live format.
- **Free access to Annual Meeting recorded events** for Annual Meeting attendees and discounted access for other Members.
- **Networking opportunities** and, for Students & Trainees, access to Mentors.
- **Opportunity to enhance your leadership skills** by volunteering for one of ACCP's many committees or by Mentoring Students & Trainees.

- **Opportunity to develop educational activities** that make a difference by submitting proposals for ACCP educational events and getting involved in the clinical pharmacology community.
- **Access to the ACCP Job Center** to view jobs and post your resume.
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- **Receipt of routine updates from ACCP** about developments in the field of clinical pharmacology and future ACCP events.

ACCP membership runs on a calendar year, January to December. Dues renewal notifications are sent in September for the coming year. Persons joining between May 1<sup>st</sup> and July 31<sup>st</sup> pay a reduced half-year fee for the current calendar year. Please note that the half-year option is only available the first year of ACCP membership. All future payments must be full-year payments. Persons joining for the first time as of August 1<sup>st</sup> pay for the coming full calendar year dues and receive August – December of the current year at no cost.

**BEFORE YOU APPLY FOR MEMBERSHIP, PLEASE NOTE IF ANY OF THE FOLLOWING PERTAIN TO YOU AND CONTACT [KLevy@ACCP1.org](mailto:KLevy@ACCP1.org) FOR EXISTING LOGIN CREDENTIALS:**

- Been a Member of ACCP in the past;
- Have attended an ACCP Annual Meeting;
- Presented a poster at an ACCP Annual Meeting;
- Participated as Faculty at an ACCP Annual Meeting.

### How to Join ACCP

ACCP has several categories of membership, please join using the membership category that is most appropriate for you.

**Please note:** A membership application is not considered complete until all required documents have been submitted and acknowledged by the ACCP Executive Office and dues have been paid. All applications must be submitted in full 30 days before the Board of Regents Meetings, the dates of which are noted below:

- February 12, 2017
- May 7, 2017
- September 16, 2017

Persons interested in becoming a Fellow should join as a Member and notify [KLevy@ACCP1.org](mailto:KLevy@ACCP1.org) about their interest in becoming a Fellow.

# Students & Trainees

## Annual Meeting Events for Students & Trainees

Student & Trainee membership and participation in ACCP's Annual Meeting are strongly encouraged and are beneficial on several levels:

- Mentoring and expert guidance
- Student & Trainee-specific events at the Annual Meeting
- Substantially discounted registration fees for educational programs
- ACCP Student Abstract Awards Program

## Student & Trainee-specific Events

Panel Discussion, Podium Presentations, Student Networking Reception and Poster Tours

On Sunday, September 25<sup>th</sup>, the following events will be hosted:

- **Panel Discussion on Career Guidance** (2:00 – 3:30 pm, White Flint Amphitheater) – A select group of ACCP Mentors whose careers have spanned various settings and disciplines within the field of clinical pharmacology will share their experiences and answer your questions in a relaxed, intimate atmosphere. If you are considering a career that includes any combination of academia, industry, regulatory or clinical roles, don't miss this opportunity to hear what the experts have to say about how their own career paths progressed and what guidance they can provide to ensure your personal success!
- **Podium Presentations** (3:30 – 4:30 pm, White Flint Amphitheater) – Immediately following the Panel Discussion, a select number of Student Abstract Award winners will present their research in a Podium Presentation to an audience of Annual Meeting attendees. Support your colleagues by being part of this important event.
- **Student Networking Reception** (4:30 – 5:30 pm, Brookside Foyer) – After the Podium Presentations, join us for the Student Networking Reception where you can interact on a more personal level with Panel Discussion speakers and other ACCP Mentors to ask the burning questions that will help you make decisions about your future.
- **Poster Tours** (Meet at ACCP Registration Desk at 5:30 pm for a tour from 5:45 – 6:30 pm) – Small groups of Students & Trainees will be hosted by an ACCP Fellow or senior Member to tour the poster area and discuss preselected posters that provide exceptional educational content or presentation.

## Special Access to the Experts

On Tuesday, September 27<sup>th</sup>, from 7:00 – 8:00 am in Ballroom Salon E – H, schools represented by groups of six or more Students & Trainees will be provided with a higher level of access to ACCP leadership. Select ACCP leaders will have a sit-down roundtable session with those Students & Trainees to discuss opportunities for further involvement in ACCP during their training and how to subsequently grow in the organization throughout their careers.

## CV Reviews!

All Students & Trainees were encouraged to provide their CV for review and suggestions by ACCP Mentors. If you submitted a resume and did not make arrangements in advance, but wish to meet with the Mentor who reviewed your CV, please stop by the ACCP Registration Desk by the end of the day on Sunday, September 25<sup>th</sup>, to set up an appointment.

## Join, Get Involved and Enjoy the Benefits of ACCP Membership!

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The **Student, Trainee & Young Professional Committee**, co-chaired by Daniel Gonzalez, PharmD, PhD and Amelia N. Deitchman, PharmD, is critical in providing guidance regarding Student, Trainee & Young Professional needs and ensuring that those needs are consistently met by ACCP. The committee is comprised of Student Members, Members and Fellows and it focuses on activities at the Annual Meeting and provides guidance on programs, new and old, required to effectively support Students, Trainees & Young Professionals. Have a great idea? Please share it with us at [SOC@ACCP1.org](mailto:SOC@ACCP1.org).



Amelia N. Deitchman, PharmD



Daniel Gonzalez, PharmD, PhD



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- ▶ Visit Booth #303
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## Poster Session 1

Sunday, September 25, 2016 / 5:30 – 7:30 pm, Ballroom Salon E – H

### Absorption, Distribution, Metabolism & Elimination

Poster	Type	Title	Authors
001	S, SA, P	Impact of Coadministered Minocycline on Plasma Pharmacokinetics and Central Nervous System Distribution of Riluzole	Mahua Sarkar, Raymond Grill, Robert Grossman, Diana Chow
002		Effect of BI 187004, a Selective 11 $\beta$ -HSD1 Inhibitor, on Cytochrome P450 and P-glycoprotein Substrates in Healthy Subjects	John P. Sabo, Fabian Mueller, Arvid Jungnik, Habib Esmaeili, Ralf Thiedmann, Jing Wu, Lois Rowland, Susanna Freude, Laurent Vernillet
003	S, NM	Biliary Excretions of CZ48 and Its Active Moiety, Camptothecin, After Intravenous Administration of CZ48 in Rats	Yu Jin Kim, Yifan Tu, Daping Zhang, Diana Chow
004		Absolute Oral Bioavailability of Selexipag, a Novel Oral Prostacyclin IP Receptor Agonist, in Healthy Subjects	Noémie Hurst, Priska Kaufmann, Muriel Richard, Béatrice Astruc, Jasper Dingemanse
005		A Change in Posture Significantly Affects Plasma Concentrations of Immunoglobulin G, Such as Monoclonal Antibodies	Mattheus (Thijs) P. van Iersel, Maria Velinova, Ruud Lutgerink
008	S, NM	Does Gastric Bypass Surgery Affect Bioavailability of Orally-administered Darunavir? A Physiologically-based Pharmacokinetic Modeling Approach	Jihye Ahn, Bilal AbuAsal, Neha Pandit, Mathangi Gopalakrishnan

### Clinical Pharmacokinetics & Pharmacodynamics (cont on next page)

Poster	Type	Title	Authors
009	S, NM	Stabilization of Pitavastatin and Its Lactone Metabolite in Clinical Pharmacokinetic Study Samples	Kristina M. Brooks, Raul Alfaro, David Ng, Jomy George, Tara Kuhn, Leslie G. Biesecker, Parag Kumar
010		Population Pharmacokinetics of Lopinavir/Ritonavir in Mexican Patients Infected with HIV	Miriam D. Carrasco-Portugal, Maria G. Lozoya-Moreno, Gustavo Reyes-Terán, Lina M. Barranco-Garduño, Francisco J. Flores-Murrieta
011		Pharmacokinetics and Safety of NSI-189 in Healthy Subjects: First-in-Human Single, Ascending Dose and Food Effect Study	Ronald Christopher, Lev Gertsik, Molly Sherman, Karl Johe, Larry Ereshefsky
012	NM	The Relative Bioavailability of Rilpivirine Following Administration of the New Tenofovir Alafenamide Single-tablet Regimen Rilpivirine/Emtricitabine/Tenofovir Alafenamide vs Rilpivirine/Emtricitabine/Tenofovir Disoproxil Fumarate	Joseph M. Custodio, Steve West, Heena Patel, Kah Hing J. Ling, Huyen Cao, Erin Quirk, Brian P. Kearney

#### LEGEND:

E = Encore Presentation  
NM = New Member (*Dues paid by April, 2016*)  
P = Podium Presentation  
S = Student Abstract  
SA = Student Award Winner

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## 2016

### Abstract Reviewers

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# Poster Session 1

Sunday, September 25, 2016 / 5:30 – 7:30 pm, Ballroom Salon E – H

## Clinical Pharmacokinetics & Pharmacodynamics (cont on next page)

Poster	Type	Title	Authors
013	S, SA	Potential Implications of Atypical, Nonlinear Plasma Protein Binding on Tigecycline Clinical Pharmacokinetics	Amelia N. Deitchman, Ravi S. Singh, Johannes Kast, Uwe Liebchen, Hartmut Derendorf
014	S, SA, P	Impact of Gastric Bypass Surgery on the Pharmacokinetics and Pharmacodynamics of Simvastatin, Atorvastatin and Rosuvastatin	Asma El-Zailik, Lily K. Cheung, Vadim Sherman, Diana Chow
015		Pharmacokinetics of Oral Tizanidine, a CYP1A2 Substrate, in Mexicans: Evidence for Interethnic Variability	Francisco J. Flores-Murrieta, Miriam D. Carrasco-Portugal
016	S	Population Pharmacokinetic Analysis of Mycophenolate Mofetil in Pre-transplant and Post-transplant Pediatric Patients	Rodrigo Gonzalez, Alan Quiroz-Moguel, Pilar Garcia, Mara Medeiros, Gilberto Castaneda, Hartmut Derendorf
017		Effect of Intrinsic Factors on the Exposure of Faldaprevir in HCV-infected Patients: Pooled Analysis of Data from Three Faldaprevir Phase 3 Studies	Fenglei Huang, Sebastian Haertter, Anne-Marie Quinson
018		Assessment of the Effect of Faldaprevir on the QT Interval in Healthy Subjects	John P. Sabo, Michael Koenen-Bergmann, Anna Unseld, Armin Schultz, Fenglei Huang, Anne-Marie Quinson
019		Effect of Food on the Pharmacokinetics of a 150 mg Oral Dose of BI 1060469, a Novel Oral CRTH2 Antagonist, in Healthy Male Volunteers	Klaus Peter Kammerer, Peter Ruus, Simone Graeber, David Joseph, Verena Endriss, Alison Mackie, Chester Wood
020		The Pharmacokinetics, Pharmacodynamics, Safety, Tolerability and Food Effect of Duvelisib, an Oral, Dual PI3K- $\delta$ and PI3K- $\gamma$ Inhibitor, in Healthy Human Subjects	Jahnavi Kharidia, Mattheus (Thijs) P. van Iersel, Jan Hartstra, Kerstin Allen, Charlotte McKee, Joi Dunbar
021		Safety and Pharmacokinetics of BI 1060469, a Novel Oral CRTH2 Antagonist, in Fed Healthy Females	Peter Ruus, Irene Vroegrijk, David Joseph, Verena Endriss, Alison Mackie, Chester Wood
022	S, NM	Comparative Pharmacokinetic Profiling of Various Polymyxin B Components	Pooja Manchandani, Yanina Dubrovskaya, Song Gao, Vincent H. Tam
023		Pharmacokinetics and Safety of TAK-648 Following Single and Repeat Dosing in Healthy and Type 2 Diabetes Mellitus Subjects	Michael D. Mayer, Juliana Oliveira, Stephanie Moran, Sai Nudurupati, Rui Sun
024	S, NM	Population Pharmacokinetics of Veliparib With and Without Topotecan Plus Carboplatin in Patients With Hematological Malignancies	Shailly Mehrotra, Mathangi Gopalakrishnan, Jogarao V. Gobburu, Jacqueline M. Greer, Ivana Gojo, Judy Karp, Keith Pratz, Michelle A. Rudek
026		Effect of Albiglutide on Cholecystokinin-induced Gallbladder Emptying in Healthy Subjects	Bonnie Shaddinger, Malcolm Young, Julia Billiard, David A. Collins, Nandana Prabhu, Azra Hussaini, Antonio Nino
027	NM	Pharmacokinetic and Pharmacodynamic Modeling of Peptide YY <sub>3-36</sub> in Mice	Jie Shao, Mong-jen Chen, Philip J. Kuehl, David Vodak, Guenther Hochhaus
028		Bioequivalence Evaluation of Three Olanzapine-containing Tablet Formulations in Healthy Volunteers	Lei Sun, David McDonnell, Wenlei Liu, Denise Carter, Adam Simmons, Lisa L. von Moltke
029		Plasma Pharmacokinetic Profile of Trabectedin – TGF- $\beta$ 2-specific Antisense Oligonucleotide in Cancer Patients	Wen Wang, Larn Hwang
030		Safety and Pharmacokinetics of Multiple Rising, Oral Doses of BI 1060469, a Novel CRTH2 Antagonist, in Fasted Healthy Males and Males with Mild Asthma	Peter Ruus, Irene Vroegrijk, David Joseph, Verena Endriss, Alison Mackie, Chester Wood

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## Poster Session 1

Sunday, September 25, 2016 / 5:30 – 7:30 pm, Ballroom Salon E – H

### *Clinical Pharmacokinetics & Pharmacodynamics*

Poster	Type	Title	Authors
031		Safety, Tolerability, Pharmacokinetics and Pharmacodynamics of Single and Multiple, Oral Doses of BI 187004, a Selective 11 $\beta$ -hydroxysteroid Dehydrogenase1 Inhibitor, in Healthy Subjects and Patients With Type 2 Diabetes Mellitus	Jing Wu, Valerie Nock, Arvid Jungnik, Corinna Schoelch, Michael Wolff, Susanna Freude, Cornelia Schepers, John Yu, Tim Heise, Leona Plum-Moerschel, Fenglei Huang, Laurent Vernilliet
032		Population Pharmacokinetics of Canakinumab in Children Younger Than Two Years Old With Cryopyrin-associated Periodic Syndrome	Lucy Xu, Bruno Bieth, Martin Fink, James Kalabus, Haiying Sun

### *Clinical Pharmacology Education*

Poster	Type	Title	Authors
033		Some Thoughts About the Mean Concentration vs Time Plot	Michael J. Fossler, Jr

### *Clinical Trials & Human Pharmacology*

Poster	Type	Title	Authors
034	S	FPGS rs1544105 and rs4451422 are Involved With High Levels of Plasmatic Concentration of Methotrexate in Mexican Children With Acute Lymphoblastic Leukemia	Fausto Zaruma-Torres, Ismael Lares-Asseff, Juan L. Chávez-Pacheco, Aarón Reyes-Espinoza, Ossyneidee Gutiérrez-Alvarez, Verónica Loera-Castañeda, María C. Arias-Peláez
035		Cardiac Safety and Pharmacokinetics of Pacritinib: A Phase 1, Randomized, Active- and Placebo-controlled, Three-way Crossover Study	Suliman Al-Fayoumi, Sherri Amberg, Huafeng Zhou, Lindsey Millard, Jack W. Singer, James P. Dean
036		Pharmacokinetics, Pharmacodynamics and Tolerability of Multiple-dose Administration of Cenerimod, a Selective Sphingosine-1-phosphate Subtype 1 Receptor Modulator	Pierre-Eric Juif, Jasper Dingemanse
037		Pharmacokinetics, Pharmacodynamics, Tolerability and Food Effect of Single-dose Administration of Cenerimod, a Selective Sphingosine-1-phosphate Subtype 1 Receptor Modulator, in the First-in-Human Study	Pierre-Eric Juif, Jasper Dingemanse
038		Profiling Adverse Events and Laboratory Measurements in Healthy Volunteers Who Received Placebo in AbbVie Phase 1 Trials	Hong Li, Jack Clifton II, Mukul Minocha, David Carter, Ahmed A. Othman, Yi-Lin Chiu
039		A Phase 1 Dose-escalation Study of the Safety and Pharmacokinetics of Felbinac Trometamol in Healthy Chinese Volunteers	Lihua Wu, Jian Liu, Jianzhong Shentu, You Zhai, Guolan Wu, Xingjiang Hu, Yunliang Zheng, Meihua Lin, Duo Lv, Juan Xu, Huili Zhou, Meixiang Zhu, Minglan Wu

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# Poster Session 1

Sunday, September 25, 2016 / 5:30 – 7:30 pm, Ballroom Salon E – H

## Oncology

Poster	Type	Title	Authors
040	S, SA	Development and Evaluation of Tri-functional Lipid Nanoparticles for Treatment of HER2-positive Breast Cancer Refractory to HER2 Therapy	Tanaya R. Vaidya, Sihem Bihorel
041	S, SA, NM, P	Failure of Intravenous Busulfan Test Dose Pharmacokinetics to Predict Once-daily Dosing in Pediatric Transplant Recipients	Kristina M. Brooks, Paul Jarosinski, Elizabeth Kang, Jomy George, Nirali N. Shah, John B. Le Gall, Suk See De Ravin, Thomas Hughes, Parag Kumar
042		Echocardiographic Evaluation of Cardiotoxic Drugs: Is Ejection Fraction Adequate?	Gerald H. Sokol, Loretta S. Loftus, Jorge Ayub, Louis Cantilena
043	S	Preclinical Evaluations of Two Vascular Endothelial Growth Factor Inhibitors in Combination With Rapamycin for the Treatment of Hepatocellular Carcinoma	Maher Chaar, Ferraro Matthew, Erica Johnson, Maxime Le Merdy, Ashley Brown, Sihem Bihorel
044	NM	Model-based Evaluations of the Exposure, Efficacy and Safety of a Nivolumab Flat-dosing Regimen in Patients With Melanoma or Non-small Cell Lung Cancer	Xiaochen Zhao, Satyendra Suryawanshi, Yan Feng, Xiaoning Wang, Brent McHenry, Ian M. Waxman, Anand Achanta, Akintunde Bello, Amit Roy, Shruti Agrawal

## Translational Medicine, Including Biomarkers and/or Imaging

Poster	Type	Title	Authors
045	NM	Intra-target Microdosing, A Novel Drug Development Approach: Proof-of-Concept in Humans	Tal Burt, David MacLeod, Kihak Lee, Thomas Hawk, Timothy Turkington, Antoinette Santoro, Daniel K. DeMasi, Mark Feinglos, Robert Noveck, Malcolm Rowland

## Chronic Pain Management

Poster	Type	Title	Authors
057		Randomized, Double-blind, Placebo- and Comparator-controlled Human Abuse Liability Study of an Experimental, Triple Monoamine Reuptake Inhibitor in Recreational Drug Abusers	Lynn R. Webster, Michael Smith

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## Poster Session 2

Monday, September 26, 2016 / 5:30 – 7:30 pm, Ballroom Salon E – H

### Applications of Modeling & Simulation

Poster	Type	Title	Authors
046		Application of a Physiologically-based Pharmacokinetic Model for the Evaluation of Single-point Plasma Phenotyping Method of CYP2D6	Rui Chen, Amin Rostami-Hodjegan, Haotian Wang, David Berk, Jun Shi, Pei Hu
047		Comparative Application of Published Top-down and Bottom-up Pharmacokinetic Models of Efavirenz With Respect to Observed Ethiopian Clinical Data	Abiy Habtewold, Andrew Castleman, Joel S. Owen
048		Development of a Physiologically-based Pharmacokinetic Model for Description of Valganciclovir and Ganciclovir Pharmacokinetics	Viera Lukacova, Petra Goelzer, Micaela Reddy, Gerard Greig, Bruno Reigner, Neil Parrott
050		Simulating Altered Omeprazole Kinetics in Elderly Individuals Using Physiologically-based Pharmacokinetic Population Modeling	Jan-Frederik Schlender, Niels Lautenbach, Tobias Kanacher, Michael Block, Thomas Eissing
051		Physiologically-based Pharmacokinetic Modeling to Predict the Human Pharmacokinetics of Olanzapine and Samidorphan When Administered in Combination as ALKS 3831	Lei Sun, Karen Rowland Yeo
052		Nonlinear Mixed-effects Pharmacokinetic-Pharmacogenetic Model of Intravenously-administered Delta-9-tetrahydrocannabinol in Healthy Volunteers	William R. Wolowich, Leah Bensimon, Robert Greif, Maren Kleine-Bruggeney, Hans Sachs, Werner Bernhard, Lorenz Theiler
053	S	Population Pharmacokinetics of Unbound Mycophenolic Acid in Pediatric and Adolescent Patients Post-hematopoietic Stem Cell Transplantation	Daping Zhang, Diana Chow, Jamie L. Renbarger

### Biosimilars

Poster	Type	Title	Authors
054	E	Comparison of Metabolic and Mitogenic Response <i>In Vitro</i> of the Rapid-acting Insulin Lispro Product SAR342434 and US- and EU-approved Humalog®	Juergen Dedio, Birgit Niederhaus, Marcus Korn, Surya Prakash, Norbert Tennagels
055	E	Use of an <i>In Vitro</i> Model of Human Immunity to Evaluate the Innate Immune Profile of Originator and Biological Copies of Insulin Glargine	Ernesto Luna, Pankaj Agrawal, Riyaz Mehta, Maria E. Boone, Charlotte Vernhes, Colombe Denys, Bhaswati Mukherjee, Norbert Tennagels, Donald Drake

### Decision Making in Research & Development

Poster	Type	Title	Authors
058		Use of an Obese Population in Phase 1 to Evaluate the Pharmacology of Oral CXA-10, an Endogenous, Nitro-fatty Acid Signaling Agent	Carla Chieffo, Jeff Botbyl, Kim Perry, Thomas M. Blok, Diane K. Jorkasky
059		Placebo as Gold Standard in Randomized, Controlled Trials Based on Literature Search: Potential Confounders and Ethical Concerns	Charles Oo

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## Poster Session 2

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### Drug Interactions

Poster	Type	Title	Authors
060	E	Effects of Multiple-dose Administration of Itraconazole on the Single-dose Pharmacokinetics of Conjugated Estrogens/Bazedoxifene in Postmenopausal Women	Carol Cronenberger, Anna Plotka, Kelly Ryan, Joanne Salageanu, William McKeand
061	S, SA	Communication of Drug Interactions with Gastric Acid-reducing Agents: A Survey of Clinical Practice Guidelines	Edwin Lam, Sue Chih Lee
062	NM	The Effect of Milk Thistle ( <i>Silybum marianum</i> ) and its Main Flavonolignans on CYP2C8 Enzyme Activity in Human Liver Microsomes	Ahmed A. Albassam, John S. Markowitz, Reginald F. Frye
063	S	Levo-tetrahydropalmatine Does Not Affect the Pharmacokinetics of Concomitantly-administered Cocaine in Rats	Shamia Faison, William D. Hedrich, Robert Gharavi, Hongbing Wang, Hazem Hassan
064	E	Effect of Steady-state Esuberaprost on the Safety, Pharmacokinetics and Pharmacodynamics of Warfarin in Healthy Adult Subjects	Gina Patel, Hugh Coleman, Xin Chen, Marni Peterson, Kirby von Kessler, Jordan Shin, Prakash Sista

### Experimental Pharmacology in In Vitro & In Vivo Studies

Poster	Type	Title	Authors
065		Defining Endothelin-B Receptor-mediated Neurogenesis in Mice Tolerant to Opioids	Shruti Gulati, Shantel Jones, Seema Briyal, Anil Gulati, Shaifali Bhalla
066		Prenatal Exposure to Ethanol and Oxycodone Affects Neurogenesis and Impairs Development of the Neonatal Rat Brain	Shruti Gulati, Seema Briyal, Mary Leonard, Muhammad Ansari, Muralidhara Devarapalli, Lorene Schweig, Bhagya Puppala, Anil Gulati
067		Extended Therapeutic Window for Neuroprotection by IRL-1620 in the Treatment of Cerebral Ischemia	Ahmed Maki, Seema Briyal, Anil Gulati
068		Effect of Maternal Cannabinoid Abuse on CB1, ETB, VEGF and NGF Expression in the Postnatal Rat Brain	Kevin Cooper, Mary Leonard, Seema Briyal, Aarti Amlani, Preetha Prazad, Ramona Donovan, Anil Gulati
069	NM	Effect of Tidal Volume on Cardiovascular and Blood Gas Parameters in a Lipopolysaccharide Infusion Model of Septic Shock in the Rat	Gwendolyn Pais, Zhong Zhang, Suresh Havalad, Anil Gulati
070		In Vitro Pharmacologic Characteristics of Valbenazine and its Metabolites	Dimitri Grigoriadis, Evan Smith, Ajay Madan, Bill Aurora, Haig Bozigian

### Mechanism of Action

Poster	Type	Title	Authors
071	S, NM	Development of Dependence After Exposure to Cannabis Smoke in Rats	Abhigyan Ravula, Adriaan Bruijnzeel, Barry Setlow, Marcelo Febo, Darin Jagarine, Hartmut Derendorf

### Model-based Drug Development

Poster	Type	Title	Authors
072	NM	Population Pharmacokinetics and Exposure-Response Modeling Analyses of Golimumab in Pediatric Patients With Moderate-to-Severe Ulcerative Colitis	Yan Xu, Omoniyi Adedokun, Daphne Chan, Chuanpu Hu, Zhenhua Xu, Richard Strauss, Honghui Zhou

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## Poster Session 2

Monday, September 26, 2016 / 5:30 – 7:30 pm, Ballroom Salon E – H

### Pharmacogenomics

Poster	Type	Title	Authors
074		Analysis of the CYP2D6*10 Allele in Relation to Dextromethorphan O-demethylation Capacity in a Chinese Population	Rui Chen, Xin Zheng, Jun Shi, Pei Hu

### Pharmacometrics

Poster	Type	Title	Authors
076	S, SA, P	To Antidote or Not? Web-based Antidote Recommendation Tool for Acute Acetaminophen Overdose	Jessica Wojciechowski, Julie Desrochers, Wendy Klein-Schwartz, Suzanne Doyon, Jogarao V. Gobburu, Mathangi Gopalakrishnan
077		Population Pharmacokinetic and Pharmacodynamic Modeling of Glucalpidase and High-dose Methotrexate Using Modified Michaelis-Menten Elimination Model	Toshimi Kimura, Hiroshi Kawamoto, Yutaka Fukaya, Shinobu Kashiwase, Mariko Takahashi
078		Voriconazole Dosing in Children Under Two Years	Michael Neely, Xiaowei Fu, Ashley Margol, Teresa Rushing, Siyu Liu, Stan Louie
079	E	Disease-Drug Interaction of Sarilumab and Simvastatin in Patients With Rheumatoid Arthritis	Eun B. Lee, Nikki Daskalakis, Christine Xu, Anne Paccaly, Barry Miller, Roy Fleischmann, Inga Bodrug, Alan Kivitz
080		Model-based Meta-analysis of Progression-free Survival Time in Non-Hodgkin's Lymphoma Patients	Mengyao Li, Ahmed Hamed Salem, Shekman Wong, Kevin Freise

### Regulatory Issues

Poster	Type	Title	Authors
081		Is Generic Tacrolimus Bioequivalent In Patients?	Alfred H. Balch, Tian Yu, Joseph E. Rower, Joseph R. Sherbotie, E.K. Korgenski, Catherine M.T. Sherwin
082	NM	Impact of Variability on Therapeutic Success for Drugs with Narrow Therapeutic Index	Elyes Dahmane, Mathangi Gopalakrishnan, Liang Zhao, Lanyan Fang, Jogarao V. Gobburu, Vijay Ivaturi
083	NM	A Signal-to-Noise Ratio Classification System of Drugs to Investigate Generic Drug Ineffectiveness Claims	Eliford N. Kitabi, Vijay Ivaturi, Lanyan Fang, Liang Zhao, Jogarao V. Gobburu, Mathangi Gopalakrishnan
084		Evaluation of QT Variation Over 24 h: Correlation With Food Intake	Jörg Täubel, Dilshat Djumanov, Sara Fernandes, Georg Ferber
085		Stability of the Effect of a Standardized Meal on QTc	Jörg Täubel, Sara Fernandes, Georg Ferber

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### *Risk Management, Legal Issues*

Poster	Type	Title	Authors
086		Risk-based Monitoring: A Global Study Focusing on Perception and Merits Among Clinical Investigational Sites	Prajna Kumar, Manoj P. Jadhav

### *Safety & Efficacy*

Poster	Type	Title	Authors
087		QT Correction For Heart Rate: Use of the Absolute Correction Factor	Charles Oo, Thomas Chou, Michael J. Fossler, Jr

### *Special Populations, Including Women, Children, the Elderly & Obese Patients*

Poster	Type	Title	Authors
088	S, SA, P, NM	Dose Optimization of Dichloroacetate Using a Semi-mechanistic Population Pharmacokinetic Model	Naveen Mangal, Albert Shroads, Taimour Langae, Peter Stacpoole, Stephan Schmidt
089	S, SA	Development and Qualification of a Pediatric Population-based Pharmacokinetic Model for Biologics in PK-Sim®	Sumit Basu, Christoph Niederaalt, Haitao Yang, Yi Ting Lien, Thomas Eissing, Stephan Schmidt
090	S	Vancomycin-associated Nephrotoxicity Among Pediatric Patients With Hematologic Malignancy	Rose Caston, Jonathan E. Constance, Alfred H. Balch, E.K. Korgenski, Catherine M.T. Sherwin
091	S	Pharmacokinetics of Clofarabine in Pediatric Subjects Prior to Allogeneic Hematopoietic Cell Transplantation	Aksana K. Jones, Vijay Ivaturi, Janel Long-Boyle
092		Prediction of Valganciclovir and Ganciclovir Pharmacokinetics in Different Pediatric Groups Using a Physiologically-based Pharmacokinetic Model	Viera Lukacova, Petra Goelzer, Micaela Reddy, Gerard Greig, Bruno Reigner, Neil Parrott
093		Further Validation of a Novel Descriptor of Renal Drug Elimination in Neonates Using Teicoplanin	Virginia Ramos-Martin, Walter Yamada, Michael Neely
094	E	A Comparison of Safety and Pharmacokinetics of Esuberaprost (BPS-314d-MR) in Subjects with Normal, Mild and Moderate Hepatic Impairment	Gina Patel, Thomas Marbury, Kenneth Lasseter, Jolene K. Berg, Xin Chen, Stephanie Peychal, Kirby von Kessler, Jordan Shin, Prakash Sista
095		Venetoclax Pharmacokinetics in Hematological Malignancies Subjects with Renal and Hepatic Impairment	Aksana Jones, Kevin Freise, Suresh Agarwal, Maria Verdugo, Rod Humerickhouse, Shekman Wong, Ahmed Hamed Salem

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## New Members: August 1, 2015 – July 31, 2016

Oluseyi Adeniyi, PharmD, PhD\*\*  
Jihye Ahn, MS\*\*  
Ahmed A. Albassam, PhD  
Celina Alvarez, BS  
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Vijay Ivaturi, MS, PhD  
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Hechuan Wang, MS\*\*  
Yan Xu, MD, PhD  
Ryoichi Yano, PhD  
Zhiling Yu, PhD\*\*  
Shadia Zaman, PhD\*\*  
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Yilong Zhang, PhD  
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\* Fellow

\*\* Student Member

**American College of Clinical Pharmacology**

21750 Red Rum Dr, Suite 137, Ashburn, VA 20147

MAILING ADDRESS: PO Box 1758, Ashburn, VA 20146-1758

PHONE: 571.291.3493

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